As Filed with the
Securities and Exchange Commission on June 7, 2019.

Registration No. 333-231700

UNITED STATES
SECURITIES AND EXCHANGE COMMISSION
Washington, D.C. 20549

AMENDMENT NO. 1
to
FORM S-1
REGISTRATION STATEMENT
UNDER
THE SECURITIES ACT OF 1933

STOKE THERAPEUTICS, INC.

(Exact name of registrant as specified in its charter)

Delaware	**2834**	**47-1144582**
(State or other jurisdiction of incorporation or organization)	**(Primary Standard Industrial Classification Code Number)**	**(I.R.S. Employer Identification Number)**

45 Wiggins Avenue
Bedford, MA 01730
(781)-430-8200
(Address, including zip code, and telephone number, including area code, of registrant's principal executive offices)

Edward M. Kaye, M.D.
Chief Executive Officer
Stoke Therapeutics, Inc.
45 Wiggins Avenue
Bedford, MA 01730
(781)-430-8200
(Name, address, including zip code, and telephone number, including area code, of agent for service)

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Approximate date of commencement of proposed sale to the public: As soon as practicable after the effective date of this registration statement.

If any of the securities being registered on this form are to be offered on a delayed or continuous basis pursuant to Rule 415 under the Securities Act of 1933 check the following box. ☐

If this form is filed to register additional securities for an offering pursuant to Rule 462(b) under the Securities Act, check the following box and list the Securities Act registration statement number of the earlier effective registration statement for the same offering. ☐

If this form is a post-effective amendment filed pursuant to Rule 462(c) under the Securities Act, check the following box and list the Securities Act registration statement number of the earlier effective registration statement for the same offering. ☐

If this form is a post-effective amendment filed pursuant to Rule 462(d) under the Securities Act, check the following box and list the Securities Act registration number of the earlier effective registration statement for the same offering. ☐

Indicate by check mark whether the registrant is a large accelerated filer, an accelerated filer, a non-accelerated filer, a smaller reporting company, or and "emerging growth company". See the definitions of "large accelerated filer," "accelerated filer," "smaller reporting company," and "emerging growth company" in Rule 12b-2 of the Exchange Act:

Large accelerated filer ☐	Accelerated filer ☐
Non-accelerated filer ☐	Smaller reporting company ☒
	Emerging growth company ☒

If an emerging growth company, indicate by check mark if the registrant has elected not to use the extended transition period for complying with any new or revised financial accounting standards provided to Section 7(a)(2)(B) of the Securities Act. ☐

CALCULATION OF REGISTRATION FEE

Title of Securities to be Registered	Amount to be Registered[1]	Proposed Maximum Offering Price Per Share	Proposed Maximum Aggregate Offering Price[2]	Amount of Registration Fee[3]
Common Stock, par value $0.0001 per share	7,705,000	$16.00	$123,280,000	$14,942

(1) Estimated solely for the purpose of calculating the amount of the registration fee pursuant to Rule 457(a) under the Securities Act of 1933, as amended. Includes 1,005,000 additional shares that the underwriters have the option to purchase.

(2) Estimated solely for the purpose of calculating the amount of the registration fee.

(3) The Registrant previously paid $10,454 of this amount in connection with the initial filing of this Registration Statement.

The Registrant hereby amends this Registration Statement on such date or dates as may be necessary to delay its effective date until the Registrant shall file a further amendment which specifically states that this Registration Statement shall thereafter become effective in accordance with Section 8(a) of the Securities Act of 1933, as amended, or until the Registration Statement shall become effective on such date as the Securities and Exchange Commission, acting pursuant to said Section 8(a), may determine.

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The information in this prospectus is not complete and may be changed. We may not sell these securities until the registration statement filed with the Securities and Exchange Commission is effective. This prospectus is not an offer to sell these securities, and we are not soliciting offers to buy these securities in any jurisdiction where the offer or sale is not permitted.

Subject to completion, dated June 7, 2019

Prospectus

6,700,000 shares

Common stock

This is the initial public offering of shares of our common stock. We are offering 6,700,000 shares of our common stock. The initial public offering price is expected to be between $14.00 and $16.00 per share.

Prior to this offering, there has been no market for our common stock. We have applied to list our common stock on the Nasdaq Global Market under the symbol “STOK.” Upon the closing of this offering, we will be a “controlled company” within the meaning of the applicable listing rules of the Nasdaq Global Market.

We are an “emerging growth company” and a “smaller reporting company” as defined under the federal securities laws and, as such, may elect to comply with certain reduced public company reporting requirements for future filings. Investing in our common stock involves a high degree of risk. Please see the section entitled “Risk Factors” starting on page 11 to read about risks you should consider carefully before buying shares of our common stock.

	Per share	Total
Initial public offering price	$	$
Underwriting discounts and commissions(1)	$	$
Proceeds to Stoke Therapeutics, Inc., before expenses	$	$

(1) We have agreed to reimburse the underwriters for certain FINRA-related expenses. See “Underwriting” for additional information regarding the compensation payable to the underwriters.

We have granted the underwriters an option for a period of 30 days to purchase up to 1,005,000 additional shares of common stock from us at the public offering price, less underwriting discounts and commissions.

Neither the Securities and Exchange Commission nor any other regulatory body has approved or disapproved of these securities or passed on the adequacy or accuracy of this prospectus. Any representation to the contrary is a criminal offense.

The underwriters expect to deliver the shares of common stock to purchasers on , 2019.

J.P. Morgan **Cowen** **Credit Suisse**

Canaccord Genuity

Prospectus dated , 2019

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Through and including , 2019 (the 25th day after the date of this prospectus), all dealers effecting transactions in these securities, whether or not participating in this offering, may be required to deliver a prospectus. This delivery is in addition to a dealer's obligation to deliver a prospectus when acting as an underwriter and with respect to an unsold allotment or subscription.

Neither we nor the underwriters have authorized anyone to provide any information or to make any representations other than those contained in this prospectus or in any free writing prospectuses we have prepared. We and the underwriters take no responsibility for, and can provide no assurance as to the reliability of, any other information that others may give you. This prospectus is an offer to sell only the shares offered hereby, but only under circumstances and in jurisdictions where it is lawful to do so. The information contained in this prospectus is accurate only as of the date of this prospectus, regardless of the time of delivery of this prospectus or of any sale of the common stock.

Persons who come into possession of this prospectus and any applicable free writing prospectus in jurisdictions outside the United States are required to inform themselves about and to observe any restrictions as to this

offering and the distribution of this prospectus and any such free writing prospectus applicable to that jurisdiction.

Trademarks and tradenames

The mark “Stoke Therapeutics” is our registered trademark. The Stoke logo and all product names are our common law trademarks. All other service marks, trademarks and tradenames appearing in this prospectus are the property of their respective owners. Solely for convenience, the trademarks and tradenames referred to in this prospectus appear without the ® and ™ symbols, but those references are not intended to indicate, in any way, that we will not assert, to the fullest extent under applicable law, our rights, or the right of the applicable licensor to these trademarks and tradenames.

Market and industry data

This prospectus contains estimates and other statistical data made by independent parties, as well as by Health Advances LLC in a report that we commissioned, and by us relating to our industry and the markets in which we operate, including our general expectations and market position, market opportunity, the incidence of certain medical conditions and other industry data. These data, to the extent they contain estimates or projections, involve a number of assumptions and limitations, and you are cautioned not to give undue weight to such estimates or projections. Although we have not independently verified the accuracy or completeness of the data contained in these industry publications and reports, based on our industry experience we believe that the publications are reliable, the conclusions contained in the publications and reports are reasonable and the third-party information included in this prospectus and in our estimates is accurate and complete. While we are not aware of any misstatements regarding the industry, survey or research data provided herein, our estimates involve risks and uncertainties and are subject to change based upon various factors, including those discussed under the sections titled “Risk factors” and “Special note regarding forward-looking statements.” These and other factors could cause results to differ materially from those expressed in these publications and reports.

Prospectus summary

This summary highlights selected information contained elsewhere in this prospectus and does not contain all of the information that you should consider in making your investment decision. Before investing in our common stock, you should carefully read this entire prospectus, including our consolidated financial statements and the related notes thereto and the information set forth under the sections entitled "Risk factors," "Selected consolidated financial data" and "Management's discussion and analysis of financial condition and results of operations," in each case included in this prospectus. Some of the statements in this prospectus constitute forward-looking statements that involve risks and uncertainties. See the section entitled "Special note regarding forward-looking statements." Unless the context otherwise requires, we use the terms "Stoke," "company," "we," "us" and "our" in this prospectus to refer to Stoke Therapeutics, Inc.

Company overview

We are pioneering a new way to treat the underlying causes of severe genetic diseases by precisely upregulating protein expression. We are developing novel antisense oligonucleotide, or ASO, medicines that target ribonucleic acid, or RNA, and modulate precursor-messenger RNA, or pre-mRNA, splicing to upregulate protein expression where needed and with appropriate specificity to near normal levels. We utilize our proprietary technology platform, Targeted Augmentation of Nuclear Gene Output, or TANGO, to design ASOs to upregulate the expression of protein by individual genes in a patient. Our approach is designed to allow us to deliver in a highly precise, durable and controlled manner disease-modifying therapies to a broad range of relevant tissues, including the central nervous system, or CNS, eye, kidney and liver. We designed our lead product candidate, STK-001, to treat Dravet syndrome, a severe and progressive genetic epilepsy. With a well-defined patient population based on routine genetic testing and learnings from recently approved drugs for the treatment of Dravet syndrome to inform the clinical and regulatory pathways for STK-001, we anticipate an efficient clinical program for STK-001. We plan to submit an investigational new drug application, or IND, for STK-001 by early 2020 and expect to initiate a Phase 1/2 clinical trial in the first half of 2020. We intend to nominate a second candidate to treat an additional genetic disease for preclinical development by the first half of 2020.

We are developing TANGO as potentially the first precision medicine platform for treating a category of severe genetic diseases known as autosomal dominant haploinsufficiencies, or diseases in which only one copy, or allele, of the gene needs to be mutated for the disease or trait to develop, and in which that mutated allele generates a protein that is severely deficient in amount or activity, resulting in approximately 50% of normal protein expression in the patient. Our novel ASOs are designed to address this protein deficiency by precisely upregulating target protein expression and have the potential to provide disease-modifying therapies to treat many diseases beyond the reach of current approaches. Within haploinsufficiencies, we are initially prioritizing the development of ASOs for the treatment of genetic epilepsies, and specifically Dravet syndrome. Current treatments for Dravet syndrome only address the occurrence of seizures. According to a 2017 study as published in the *Developmental Medicine & Child Neurology Journal*, more than 90% of Dravet syndrome patients still report suffering from incomplete seizure control with existing antiepileptic regimens. Though we are early in our development, and have only completed preclinical studies to date, we believe we are developing the first genetic medicine designed to target the underlying cause of Dravet syndrome with the potential to significantly reduce the occurrence of seizures, and also potentially address, for the first time, the severe intellectual and developmental disabilities of the disease.

Today, multiple therapeutic modalities, including gene therapy, gene editing, modified mRNA, protein-based drugs, small molecules and oligonucleotides are approved or are being developed to address all types of monogenic diseases. However, most of these therapeutic approaches are focused on autosomal recessive or

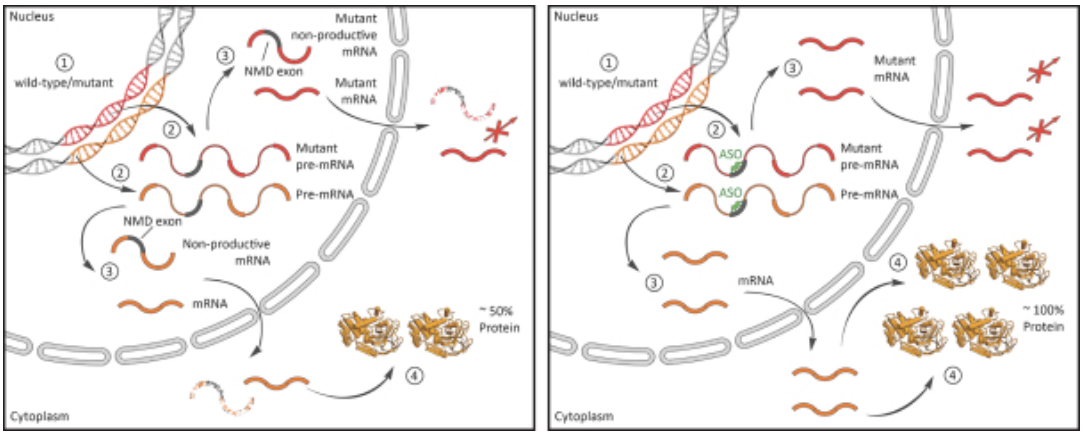
autosomal dominant gain-of-function diseases, and existing precision medicine platforms have fundamental limitations that make them poorly suited to address haploinsufficiencies. Numerous technical challenges preclude effective application of these modalities, including the inability to control level and tissue distribution of target protein expression, potential irreversible on- and off-target effects, target gene size limitations and incompatibility with diseases caused by many mutations. As a result, there is a need for novel therapeutics that can restore protein expression and address the underlying genetic causes of haploinsufficiencies.

Our precision medicine platform

Treatment of autosomal dominant haploinsufficiency diseases with TANGO

TANGO exploits unique, patented mechanisms for antisense-mediated modulation of splicing to prevent the synthesis of naturally occurring non-productive messenger RNA, or mRNA, and to increase the synthesis of productive mRNA to increase production of functional protein. TANGO is designed to address multiple forms of non-productive splicing events and is amenable to most loss-of-function mutations, thereby potentially providing a single-drug approach for diseases that are caused by a large number of mutations in a single gene. We have assembled a proprietary database of non-productive events in the human transcriptome and have identified approximately 2,900 monogenic, or single gene, diseases which we believe are amenable to TANGO. We have an intellectual property estate that includes multi-national allowed and pending claims for the TANGO mechanisms, as well as multi-national pending claims relating to compositions of matter of oligonucleotides designed to target specific TANGO elements in genes for more than 140 genetic diseases that we believe are amenable to upregulation of target protein expression using TANGO.

The figures below illustrate the TANGO mechanism for increasing protein synthesis in a prospective patient with a haploinsufficiency. To date, we have demonstrated this TANGO mechanism in preclinical models of haploinsufficiency. The left panel illustrates the prospective patient with a haploinsufficiency possessing one wild-type allele and one mutant allele. The mutant allele is translated into non-functional protein and results in approximately 50% of normal protein expression. In the right panel, treatment with our ASO would prevent the synthesis of naturally occurring non-productive mRNA and would increase the synthesis of productive mRNA, thereby restoring the target protein to near normal levels. Our preclinical studies show that any increase in mutant mRNA would have no effect on the net protein level.



Advantages of TANGO

We believe TANGO may have several key advantages, including:

- *Ability to address the underlying genetic cause of the disease.* We utilize TANGO to design ASOs to precisely upregulate protein expression, thereby addressing the underlying cause of the disease rather than the symptoms of the disease.
- *Applicability is mutation-independent.* Our ASOs upregulate expression of the wild-type allele, meaning the TANGO mechanism does not rely on targeting a specific mutation.
- *Utility across small and large gene targets encoding intracellular and extracellular proteins.* Our ASOs upregulate protein expression regardless of gene size and are not constrained to smaller gene targets.
- *No observed unwanted off-target effects.* TANGO-mediated upregulation of protein expression only occurs where the gene is being naturally transcribed, limiting the likelihood of expression in non-native tissues.
- *Ability to control dose level and duration.* Our ASOs provide the ability for dose titration, thereby allowing for dose-dependent and reversible control of level and duration of protein expression. The ability to titrate dosage provides us with flexibility to address a variety of tissue types, and potentially enables us to deliver the right dose, at the right location, for each indication.
- *Utility across a wide array of diseases and tissue types.* We believe that ASO delivery to the CNS, eye, kidney and liver is well-established, providing us the potential to address a broad range of genetic diseases. Additionally, we believe ASO delivery to the CNS is particularly well-precedented.
- *Fixed dose, rather than weight-based dosing*. For CNS and eye targets, the dose of our ASOs should not require adjustment between patients to be effective. We believe that a fixed dose across all ages in these targets will lessen reimbursement hurdles associated with a weight-adjusted dose pricing model.
- *Favorable dosing regimen.* We believe our ASOs may require as few as two to three administrations per year for the CNS or the eye and will generally involve relatively low doses, which would translate to simplified use, an improved safety profile from reduced systemic exposure and lower cost of goods.
- *Simple and scalable manufacturing*. Our novel ASOs are synthesized by highly scalable, solid-phase chemical synthesis and we leverage a well-established contract manufacturing base. We believe the manufacturing requirements for our ASOs are much simpler, more scalable and more cost-effective than gene therapy and gene editing.

Our programs

Dravet syndrome—STK-001

Our most advanced program is a potentially disease-modifying treatment for Dravet syndrome, a severe and progressive genetic epilepsy. We have generated preclinical data demonstrating proof-of-mechanism for STK-001 and intend to submit an IND by early 2020. We are leveraging similar ASO chemistry as the approved drug, SPINRAZA, which minimizes potential safety and biodistribution risks in the CNS. We applied for Orphan Drug Designation from the FDA in May 2019, and we expect to initiate a Phase 1/2 clinical trial in children and adolescents with Dravet syndrome in the first half of 2020 and anticipate clinical data, including preliminary efficacy data, in 2021. If we see evidence of efficacy following clinical data, then we would plan to meet with regulatory authorities to discuss expedited regulatory pathways, in addition to requesting Fast Track Designation and Breakthrough Therapy Designation. To date, the FDA has given no indication regarding whether our product candidate will receive Orphan Drug Designation or be permitted to use any such expedited pathway.

Additional product opportunities

We intend to nominate a second genetic disease preclinical candidate by the first half of 2020. We are also advancing several other early programs focused on multiple targets, including haploinsufficiency diseases of the CNS, eye, kidney and liver, given the potential of our ASOs to target cells in these organs, and will seek to further establish a pipeline of product candidates in the future. Additional non-epilepsy indications for which our technology may be applicable include autosomal dominant optic atrophy and autosomal dominant polycystic kidney disease.

Our strategy

We are using our proprietary TANGO technology platform to create ASOs for the treatment of severe genetic diseases. The critical components of our strategy include:

- Rapidly advance our lead program, STK-001, to clinical proof-of-concept, approval and commercialization.
- Prioritize genetic epilepsies for near-term development efforts.
- Expand our pipeline into other disease areas to fully exploit the potential of our proprietary platform.
- Maintain broad commercial rights to our product candidates.
- Continue to strengthen and expand our intellectual property portfolio.

Our team

Our executive management team has extensive collective expertise in human genetics and modulation of RNA processes using ASOs, as well as a track record of success in rare disease drug development. Our Chief Executive Officer, Edward M. Kaye, M.D., our Chief Operating Officer and Chief Business Officer, Huw M. Nash, Ph.D., and our Chief Medical Officer, Barry S. Ticho, M.D., Ph.D., FACC, bring extensive biotechnology and pharmaceutical industry experience to our team. In addition, our co-founders, Adrian R. Krainer, Ph.D. (who serves on our board of directors) and Isabel Aznarez, Ph.D. (who serves as our Vice President of Biology), offer extensive academic and research experience, including Professor Krainer's experience which led to the invention and development of SPINRAZA. Finally, our scientific and clinical advisory boards are comprised of leading experts in the fields of human genetics, pre-mRNA splicing and ASOs, and neurodevelopmental and neurodegenerative diseases.

Our funding

As of March 31, 2019, we have raised over $130 million in funding from two financing rounds, including investments from Apple Tree Partners, RTW Investments, RA Capital Management, Cormorant Asset Management, Perceptive Advisors and funds managed by Janus Henderson Investors, Redmile Group, Sphera Funds Management and Alexandria Venture Investments.

Risks affecting us

Our business is subject to a number of risks and uncertainties, including those highlighted in the section entitled "Risk factors" immediately following this prospectus summary. These risks include, among others, the following:

- We are early in our development efforts. If we are unable to develop, obtain regulatory approval for and commercialize STK-001 and our future product candidates, or if we experience significant delays in doing so, our business will be materially harmed.

- We have not tested any of our product candidates in clinical trials. Success in early preclinical studies or clinical trials may not be indicative of results obtained in later preclinical studies and clinical trials.
- Even if we complete the necessary preclinical studies and clinical trials, we cannot predict when, or if, we will obtain regulatory approval to commercialize a product candidate and the approval may be for a narrower indication than we seek.
- Certain of the diseases we seek to treat have low prevalence, and it may be difficult to identify patients with these diseases, which may lead to delays in enrollment for our trials or slower commercial revenue growth if STK-001 or our future product candidates are approved.
- We may not be successful in our efforts to use TANGO to expand our pipeline of product candidates and develop marketable products.
- Any product candidate for which we obtain marketing approval will be subject to extensive post-marketing regulatory requirements and could be subject to post-marketing restrictions or withdrawal from the market, and we may be subject to penalties if we fail to comply with regulatory requirements or if we experience unanticipated problems with our product candidates, when and if any of them are approved.
- Our success depends in part on our ability to obtain, maintain and protect our intellectual property. It is difficult and costly to protect our proprietary rights and technology, and we may not be able to ensure their protection.
- We depend on intellectual property licensed from third parties, and disputes regarding or termination of these licenses could result in loss of significant rights, which would harm our business.
- We have a limited operating history, a history of operating losses and may never achieve or sustain profitability.
- Even if we complete this offering, we will need substantial additional funds to advance development of STK-001 and our future product candidates, and failure to obtain timely funding may force us to delay, limit or terminate our product development programs, commercialization efforts or other operations.

Corporate information

We were incorporated under the laws of the State of Delaware in June 2014 under the name ASOthera Pharmaceuticals, Inc. We subsequently changed our name to Stoke Therapeutics, Inc. on May 18, 2016. Our principal executive office is located at 45 Wiggins Avenue, Bedford, Massachusetts, 01730, and our telephone number is (781) 430-8200. Our website address is www.stoketherapeutics.com. The information contained on, or that can be accessed through, our website is not part of, and is not incorporated by reference into, this prospectus. Investors should not rely on any such information in deciding whether to purchase our common stock.

Implications of being an emerging growth company and smaller reporting company

As a company with less than $1.07 billion in revenue during our last fiscal year, we qualify as an “emerging growth company” as defined in the Jumpstart Our Business Startups Act of 2012, or JOBS Act. An emerging growth company may take advantage of reduced reporting requirements that are otherwise applicable to public companies. These provisions include, but are not limited to:

- being permitted to present only two years of audited financial statements and only two years of related Management’s discussion and analysis of financial condition and results of operations in this prospectus;

- not being required to comply with the auditor attestation requirements on the effectiveness of our internal controls over financial reporting;
- not being required to comply with any requirement that may be adopted by the Public Company Accounting Oversight Board regarding mandatory audit firm rotation or a supplement to the auditor's report providing additional information about the audit and the financial statements;
- reduced disclosure obligations regarding executive compensation arrangements; and
- exemptions from the requirements of holding a nonbinding advisory vote on executive compensation and stockholder approval of any golden parachute payments not previously approved.

We may use these provisions until the last day of our fiscal year following the fifth anniversary of the completion of this offering. However, if certain events occur prior to the end of such five-year period, including if we become a "large accelerated filer," our annual gross revenues exceed $1.07 billion or we issue more than $1.0 billion of non-convertible debt in the prior three-year period, we will cease to be an emerging growth company prior to the end of such five-year period.

We have elected to take advantage of certain of the reduced disclosure obligations in the registration statement of which this prospectus is a part and may elect to take advantage of other reduced reporting requirements in future filings. As a result, the information that we provide to our stockholders may be different than you might receive from other public reporting companies in which you hold equity interests.

The JOBS Act provides that an emerging growth company can take advantage of an extended transition period for complying with new or revised accounting standards, until those standards apply to private companies. We have elected to take advantage of the benefits of this extended transition period and, therefore, we will not be subject to the same new or revised accounting standards as other public companies that are not emerging growth companies. Our consolidated financial statements may therefore not be comparable to those of companies that comply with such new or revised accounting standards. Until the date that we are no longer an emerging growth company or affirmatively and irrevocably opt out of the exemption provided by Section 7(a)(2)(B) of the Securities Act of 1933, as amended, or the Securities Act, upon issuance of a new or revised accounting standard that applies to our consolidated financial statements and that has a different effective date for public and private companies, we will disclose the date on which adoption is required for non-emerging growth companies and the date on which we will adopt the recently issued accounting standard.

We are also a "smaller reporting company," meaning that the market value of our stock held by non-affiliates plus the proposed aggregate amount of gross proceeds to us as a result of this offering is less than $700 million and our annual revenue is less than $100 million during the most recently completed fiscal year. We may continue to be a smaller reporting company after this offering if either (i) the market value of our stock held by non-affiliates is less than $250 million or (ii) our annual revenue is less than $100 million during the most recently completed fiscal year and the market value of our stock held by non-affiliates is less than $700 million. If we are a smaller reporting company at the time we cease to be an emerging growth company, we may continue to rely on exemptions from certain disclosure requirements that are available to smaller reporting companies. Specifically, as a smaller reporting company we may choose to present only the two most recent fiscal years of audited financial statements in our Annual Report on Form 10-K and, similar to emerging growth companies, smaller reporting companies have reduced disclosure obligations regarding executive compensation.

The offering

Common stock offered by us	6,700,000 shares
Common stock to be outstanding immediately after this offering	30,269,808 shares (or 31,274,808 shares if the underwriters exercise their option to purchase additional shares in full).
Option to purchase additional shares	We have granted the underwriters an option, exercisable for 30 days after the date of this prospectus, to purchase up to an additional 1,005,000 shares from us.
Use of proceeds	We estimate that the net proceeds from this offering will be approximately $91.0 million (or approximately $105.0 million if the underwriters exercise their option to purchase additional shares in full), based upon the assumed initial public offering price of $15.00 per share, which is the midpoint of the estimated price range set forth on the cover of this prospectus, after deducting the estimated underwriting discounts and commissions and estimated offering expenses. We intend to use the net proceeds that we receive in this offering to advance our lead product candidate, STK-001, through initiation of a Phase 3 clinical trial, to nominate, conduct preclinical studies for and demonstrate clinical proof of concept for additional product candidates and for general corporate purposes. See the section entitled “Use of proceeds.”
Risk factors	You should read the section entitled “Risk factors” in this prospectus for a discussion of factors to consider carefully before deciding to invest in shares of our common stock.
Controlled company	After the completion of this offering, an entity affiliated with Apple Tree Partners will beneficially own a majority of the voting power of all outstanding shares of our common stock. As a result, after the filing of our restated certificate of incorporation and the automatic termination of the amended and restated voting agreement as of the closing of this offering, we will be a “controlled company” within the meaning of the corporate governance standards of Nasdaq.
Proposed Nasdaq Global Market symbol	“STOK”

The number of shares of our common stock to be outstanding after this offering is based on (i) 892,223 shares of our common stock outstanding as of March 31, 2019 and (ii) the automatic conversion of all outstanding shares of our convertible preferred stock as of March 31, 2019 into an aggregate of 22,677,585 shares of common stock immediately prior to the completion of this offering and excludes:

- 4,034,649 shares of common stock issuable upon the exercise of options outstanding as of March 31, 2019 under our 2014 Equity Incentive Plan, or the 2014 Plan, with a weighted-average exercise price of $1.64 per share;

- 191,372 shares of common stock issuable upon the exercise of options granted after March 31, 2019 under the 2014 Plan, with a weighted average exercise price of $9.07 per share; and
- 2,910,316 shares of common stock reserved for future issuance under our stock-based compensation plans, consisting of (i) 395,316 shares of common stock reserved for future issuance under our 2014 Plan as of March 31, 2019, (ii) 2,200,000 shares of common stock reserved for future issuance under our 2019 Equity Incentive Plan, which will become effective on the date immediately prior to the date of the effectiveness of the registration statement of which this prospectus forms a part and (iii) 315,000 shares of common stock reserved for future issuance under our 2019 Employee Stock Purchase Plan, which will become effective on the date of the effectiveness of the registration statement of which this prospectus forms a part. Upon completion of this offering, any remaining shares available for issuance under our 2014 Plan will be added to the shares reserved under our 2019 Equity Incentive Plan and we will cease granting awards under our 2014 Plan. Our 2019 Equity Incentive Plan and 2019 Employee Stock Purchase Plan also provide for automatic annual increases in the number of shares reserved under the plans each year, as more fully described in "Executive compensation—Equity compensation plans and other benefit plans."

Except as otherwise indicated, all information in this prospectus assumes or gives effect to:

- the automatic conversion of all outstanding shares of our convertible preferred stock into an aggregate of 22,677,585 shares of common stock immediately prior to the completion of this offering;
- a 9.95-for-1 reverse stock split effected on June 6, 2019;
- the effectiveness of our restated certificate of incorporation and restated bylaws in connection with the completion of this offering;
- no exercise of outstanding options after March 31, 2019; and
- no exercise of the underwriters' option to purchase additional shares of our common stock.

Summary consolidated financial data

The following tables set forth our summary consolidated statements of operations and consolidated balance sheet data. The summary consolidated statements of operations data presented below for the years ended December 31, 2018 and 2017 are derived from our audited consolidated financial statements included elsewhere in this prospectus. The summary consolidated statements of operations data presented below for the three months ended March 31, 2019 and 2018 are derived from our unaudited consolidated financial statements included elsewhere in this prospectus. The following summary consolidated financial data should be read in conjunction with "Selected consolidated financial data," "Management's discussion and analysis of financial condition and results of operations" and our consolidated financial statements and related notes included elsewhere in this prospectus. Our historical results are not necessarily indicative of the results that may be expected in any future period. The summary consolidated financial data in this section are not intended to replace the consolidated financial statements and are qualified in their entirety by the consolidated financial statements and related notes included elsewhere in this prospectus.

	Three months ended March 31,		Year ended December 31,	
	2019	2018	2018	2017
	(In thousands, except share and per share amounts)			
Consolidated statements of operations data:				
Revenue	$ —	$ —	$ —	$ —
Operating expenses:				
Research and development	4,133	1,252	8,371	3,598
General and administrative	2,189	660	4,410	1,956
Total operating expenses	6,322	1,912	12,781	5,554
Loss from operations	(6,322)	(1,912)	(12,781)	(5,554)
Other income (expense):				
Interest income	580	—	270	—
Other expense, net	—	—	(10)	(4)
Total other income (expense)	580	—	260	(4)
Net loss	$ (5,742)	$ (1,912)	$ (12,521)	$ (5,558)
Net loss per share attributable to common stockholders, basic and diluted[1]	$ (6.89)	$ (2.78)	$ (17.65)	$ (8.29)
Weighted-average common shares outstanding, basic and diluted[1]	833,469	686,985	709,336	670,090
Pro forma net loss per share, basic and diluted[1]	$ (0.24)		$ (0.98)	
Weighted-average shares used in computing pro forma net loss per share, basic and diluted[1]	23,511,054		12,784,811	

(1) See Notes 2 and 11 to our audited consolidated financial statements and Note 9 to our unaudited consolidated financial statements included elsewhere in this prospectus for a description of how we compute basic and diluted net loss per share and basic and diluted pro forma net loss per share, and the weighted-average number of shares used in the computation of these per share amounts.

	As of March 31, 2019	
	Actual	Pro forma[1][2]
		(unaudited) (in thousands)
Consolidated balance sheet data:		
Cash, cash equivalents and restricted cash	$ 98,913	$ 190,070
Total assets	102,934	193,006
Working capital[3]	97,933	188,948
Total liabilities	3,353	2,410
Accumulated deficit	(31,452)	(31,452)
Total stockholders' equity	99,581	190,596

(1) The pro forma balance sheet data gives effect to (i) the automatic conversion of all outstanding shares of our convertible preferred stock as of March 31, 2019 into an aggregate of 22,677,585 shares of common stock immediately prior to the completion of this offering and (ii) the receipt of $91.0 million in net proceeds from the sale of 6,700,000 shares of common stock in this offering, based upon an assumed initial public offering price of $15.00 per share, which is the midpoint of the estimated price range set forth on the cover of this prospectus, after deducting the estimated underwriting discounts and commissions and estimated offering expenses.

(2) Each $1.00 increase (decrease) in the assumed initial public offering price of $15.00 per share, which is the midpoint of the estimated price range set forth on the cover of this prospectus, would increase (decrease) each of our pro forma cash, cash equivalents and restricted cash, working capital, total assets and total stockholders' equity by approximately $6.2 million, assuming that the number of shares offered, as set forth on the cover of this prospectus, remains the same and after deducting the estimated underwriting discounts and commissions. Similarly, each increase (decrease) of 1,000,000 shares in the number of shares of common stock offered would increase (decrease) each of our pro forma cash, cash equivalents and restricted cash, working capital, total assets and total stockholders' equity by approximately $14.0 million, assuming the assumed initial public offering price per share as set forth on the cover of this prospectus remains the same and after deducting the estimated underwriting discounts and commissions. The pro forma information is illustrative only, and we will adjust this information based on the actual initial public offering price and other terms of this offering determined at pricing.

(3) We define working capital as current assets less current liabilities. See our consolidated financial statements and related notes included elsewhere in this prospectus for further details regarding our current assets and current liabilities.

Risk factors

Investing in our common stock involves a high degree of risk. Before making your decision to invest in shares of our common stock, you should carefully consider the risks described below, together with the other information contained in this prospectus, including our consolidated financial statements and the related notes appearing at the end of this prospectus. We cannot assure you that any of the events discussed below will not occur. These events could have a material and adverse impact on our business, financial condition, results of operations and prospects. If that were to happen, the trading price of our common stock could decline, and you could lose all or part of your investment.

Risks related to product development and regulatory approval

We are early in our development efforts. If we are unable to develop, obtain regulatory approval for and commercialize STK-001 and our future product candidates, or if we experience significant delays in doing so, our business will be materially harmed.

We have invested substantially all of our efforts and financial resources in the development of TANGO and our current lead product candidate, STK-001 for the treatment of Dravet syndrome. We plan to submit an investigational new drug application, or IND, for STK-001 by early 2020. Our ability to generate product revenue, which we do not expect will occur for many years, if ever, will depend heavily on the successful development and eventual commercialization of TANGO and our product candidates, which may never occur. We currently generate no revenue from sales of any product and we may never be able to develop or commercialize a marketable product.

Each of our programs and product candidates will require preclinical and clinical development, regulatory approval in multiple jurisdictions, obtaining preclinical, clinical and commercial manufacturing supply, capacity and expertise, building of a commercial organization, substantial investment and significant marketing efforts before we generate any revenue from product sales. STK-001 and our future product candidates must be authorized for marketing by the U.S. Food and Drug Administration, or the FDA, or certain other foreign regulatory agencies, such as the European Medicines Agency, or the EMA, before we may commercialize any of our product candidates.

The success of STK-001 and our future product candidates depends on multiple factors, including:

- effective INDs and Clinical Trial Authorizations, or CTAs, that allow commencement of our planned clinical trials or future clinical trials for our product candidates in relevant territories;
- successful completion of preclinical studies, including those compliant with Good Laboratory Practices, or GLP, or GLP toxicology studies, biodistribution studies and minimum effective dose studies in animals, and successful enrollment and completion of clinical trials compliant with current Good Clinical Practices, or GCPs;
- positive results from our clinical programs that are supportive of safety and efficacy and provide an acceptable risk-benefit profile for our product candidates in the intended patient populations;
- receipt of regulatory approvals from applicable regulatory authorities;
- establishment of arrangements with third-party contract manufacturing organizations, or CMOs, for key materials used in our manufacturing processes and to establish backup sources for clinical and large-scale commercial supply;
- establishment and maintenance of patent and trade secret protection and regulatory exclusivity for our product candidates;

- commercial launch of our product candidates, if and when approved, whether alone or in collaboration with others;
- acceptance of our product candidates, if and when approved, by patients, patient advocacy groups, third-party payors and the general medical community;
- our effective competition against other therapies available in the market;
- establishment and maintenance of adequate reimbursement from third-party payors for our product candidates;
- our ability to acquire or in-license additional product candidates;
- prosecution, maintenance, enforcement and defense of intellectual property rights and claims; and
- maintenance of a continued acceptable safety profile of our product candidates following approval.

If we do not succeed in one or more of these factors in a timely manner or at all, we could experience significant delays or an inability to successfully commercialize our product candidates, which would materially harm our business. If we do not receive regulatory approvals for our product candidates, we may not be able to continue our operations.

We have not tested any of our product candidates in clinical trials. Success in early preclinical studies or clinical trials may not be indicative of results obtained in later preclinical studies and clinical trials.

Though ASOs have been evaluated by others in clinical trials, STK-001 has not been evaluated in human clinical trials, and we may experience unexpected or negative results in the future. We will be required to demonstrate through adequate and well-controlled clinical trials that our product candidates are safe and effective, with a favorable benefit-risk profile, for use in their target indications before we can seek regulatory approvals for their commercial sale. The positive results we have observed for our product candidates in preclinical animal models may not be predictive of our future clinical trials in humans, as mouse models carry inherent limitations relevant to all preclinical studies. In particular, the Dravet syndrome mouse model is more severe than the human disease and provides a shorter post-symptomatic observation period. Trial designs and results from early-phase trials are not necessarily predictive of future clinical trial designs or results, and initial positive results we may observe may not be confirmed in later-phase clinical trials. Our product candidates may also fail to show the desired safety and efficacy in later stages of clinical development even if they successfully advance through initial clinical trials. We may not be able to demonstrate a disease-modifying effect of STK-001 in our clinical trials in Dravet syndrome patients, even if we are able to demonstrate efficacy on seizure reduction. Even if our clinical trials demonstrate acceptable safety and efficacy of STK-001, the labeling we obtain through negotiations with the FDA or foreign regulatory authorities may not include data on secondary endpoints and may not provide us with a competitive advantage over other products approved for the same or similar indications.

Many companies in the biotechnology industry have suffered significant setbacks in late-stage clinical trials after achieving positive results in early-stage development and there is a high failure rate for product candidates proceeding through clinical trials. In addition, different methodologies, assumptions and applications we utilize to assess particular safety or efficacy parameters may yield different statistical results. Even if we believe the data collected from clinical trials of our product candidates are promising, these data may not be sufficient to support approval by the FDA or foreign regulatory authorities. Preclinical and clinical data can be interpreted in different ways. Accordingly, the FDA or foreign regulatory authorities could interpret these data in different ways from us or our partners, which could delay, limit or prevent regulatory approval. If our study data do not consistently or sufficiently demonstrate the safety or efficacy of any of our product

candidates, including STK-001, then the regulatory approvals for such product candidates could be significantly delayed as we work to meet approval requirements, or, if we are not able to meet these requirements, such approvals could be withheld or withdrawn. Regulatory delays or rejections may be encountered as a result of many factors, including changes in regulatory policy during the period of product development. We cannot be certain that we will not face similar setbacks.

Even if we complete the necessary preclinical studies and clinical trials, we cannot predict when, or if, we will obtain regulatory approval to commercialize a product candidate and the approval may be for a narrower indication than we seek.

Prior to commercialization, STK-001 and our future product candidates must be approved by the FDA pursuant to a new drug application, or NDA, in the United States and pursuant to similar marketing applications by the EMA and similar regulatory authorities outside the United States. The process of obtaining marketing approvals, both in the United States and abroad, is expensive and takes many years, if approval is obtained at all, and can vary substantially based upon a variety of factors, including the type, complexity and novelty of the product candidates involved. Failure to obtain marketing approval for a product candidate will prevent us from commercializing the product candidate. We have not received approval to market STK-001 or any of our future product candidates from regulatory authorities in any jurisdiction. We have no experience in submitting and supporting the applications necessary to gain marketing approvals, and, in the event regulatory authorities indicate that we may submit such applications, we may be unable to do so as quickly and efficiently as desired. Securing marketing approval requires the submission of extensive preclinical and clinical data and supporting information to regulatory authorities for each therapeutic indication to establish the product candidate's safety and efficacy. Securing marketing approval also requires the submission of information about the product manufacturing process to, and inspection of manufacturing facilities by, the regulatory authorities. Our product candidates may not be effective, may be only moderately effective or may prove to have undesirable or unintended side effects, toxicities or other characteristics that may preclude our obtaining marketing approval or prevent or limit commercial use. Regulatory authorities have substantial discretion in the approval process and may refuse to accept or file any application or may decide that our data are insufficient for approval and require additional preclinical, clinical or other studies. In addition, varying interpretations of the data obtained from preclinical and clinical testing could delay, limit or prevent marketing approval of a product candidate.

Approval of STK-001 and our future product candidates may be delayed or refused for many reasons, including:

- the FDA or comparable foreign regulatory authorities may disagree with the design or implementation of our clinical trials;
- we may be unable to demonstrate, to the satisfaction of the FDA or comparable foreign regulatory authorities, that our product candidates are safe and effective for any of their proposed indications;
- the results of clinical trials may not meet the level of statistical significance required by the FDA or comparable foreign regulatory authorities for approval;
- we may be unable to demonstrate that our product candidates' clinical and other benefits outweigh their safety risks;
- the FDA or comparable foreign regulatory authorities may disagree with our interpretation of data from preclinical programs or clinical trials;
- the data collected from clinical trials of our product candidates may not be sufficient to support the submission of an NDA or other comparable submission in foreign jurisdictions or to obtain regulatory approval in the United States or elsewhere;

- the facilities of third-party manufacturers with which we contract or procure certain service or raw materials, may not be adequate to support approval of our product candidates; and
- the approval policies or regulations of the FDA or comparable foreign regulatory authorities may significantly change in a manner rendering our clinical data insufficient for approval.

Even if our product candidates meet their safety and efficacy endpoints in clinical trials, the regulatory authorities may not complete their review processes in a timely manner, or we may not be able to obtain regulatory approval. Additional delays may result if an FDA Advisory Committee or other regulatory authority recommends non-approval or restrictions on approval. In addition, we may experience delays or rejections based upon additional government regulation from future legislation or administrative action, or changes in regulatory authority policy during the period of product development, clinical trials and the review process.

Regulatory authorities also may approve a product candidate for more limited indications than requested or they may impose significant limitations in the form of narrow indications, warnings or Risk Evaluation and Mitigation Strategies. These regulatory authorities may require precautions or contra-indications with respect to conditions of use or they may grant approval subject to the performance of costly post-marketing clinical trials. In addition, regulatory authorities may not approve the labeling claims that are necessary or desirable for the successful commercialization of our product candidates. Any of the foregoing scenarios could materially harm the commercial prospects for our product candidates and adversely affect our business, financial condition, results of operations and prospects.

Certain of the diseases we seek to treat have low prevalence, and it may be difficult to identify patients with these diseases, which may lead to delays in enrollment for our trials or slower commercial revenue growth if STK-001 or our future product candidates are approved.

Genetically defined diseases generally, and especially those for which our lead product candidate is targeted, have low incidence and prevalence. We estimate that the incidence of Dravet syndrome is approximately 1 in 15,625 births. This could pose obstacles to the timely recruitment and enrollment of a sufficient number of eligible patients into our trials, or limit a product candidate's commercial potential. Patient enrollment may be affected by other factors including:

- the ability to identify and enroll patients that meet study eligibility criteria in a timely manner for clinical trials;
- the severity of the disease under investigation;
- design of the study protocol;
- the perceived risks, benefits and convenience of administration of the product candidate being studied;
- the patient referral practices of providers; and
- the proximity and availability of clinical trial sites to prospective patients.

Our inability to enroll a sufficient number of patients with these diseases for our planned clinical trials would result in significant delays and could cause us to not initiate or abandon one or more clinical trials altogether. Enrollment delays in our clinical trials may result in increased development costs for our product candidate, which would cause the value of our company to decline and limit our ability to obtain additional financing.

Additionally, our projections of both the number of people who have Dravet syndrome, as well as the people with this disease who have the potential to benefit from treatment with our product candidate, are based on estimates derived from a market research study that we commissioned, which may not accurately identify the

size of the market for our product candidates. The total addressable market opportunity for STK-001 and our future product candidates will ultimately depend upon, among other things, the final labeling for our product candidates, if our product candidates are approved for sale in our target indications, acceptance by the medical community and patient access, drug pricing and reimbursement. The number of patients globally may turn out to be lower than expected, patients may not be otherwise amenable to treatment with our product candidates, or new patients may become increasingly difficult to identify or gain access to, all of which would adversely affect our results of operations and our business.

Moreover, in light of the limited number of potential patients impacted by Dravet syndrome, our per-patient therapy pricing of STK-001, if approved, must be high in order to recover our development and manufacturing costs, fund additional research and achieve profitability. We may also need to fund patient support programs upon the marketing of a product candidate, which would negatively affect our product revenue. We may be unable to maintain or obtain sufficient therapy sales volumes at a price high enough to justify our development efforts and our sales, marketing and manufacturing expenses.

We may not be successful in our efforts to use TANGO to expand our pipeline of product candidates and develop marketable products.

Because we have limited financial and managerial resources, we focus on research programs and product candidates that we identify for specific indications. Our business depends on our successful development and commercialization of the limited number of internal product candidates we are researching or have in preclinical development. Even if we are successful in continuing to build our pipeline, development of the potential product candidates that we identify will require substantial investment in additional clinical development, management of clinical, preclinical and manufacturing activities, regulatory approval in multiple jurisdictions, obtaining manufacturing supply capability, building a commercial organization, and significant marketing efforts before we generate any revenue from product sales. Furthermore, such product candidates may not be suitable for clinical development, including as a result of their harmful side effects, limited efficacy or other characteristics that indicate that they are unlikely to be products that will receive marketing approval and achieve market acceptance. If we cannot validate TANGO by successfully developing and commercializing product candidates based upon our technological approach, we may not be able to obtain product revenue in future periods, which would adversely affect our business, prospects, financial condition and results of operations.

Although we intend to nominate a second genetic disease candidate for preclinical development in the first half of 2020, we are primarily focused on our lead product candidate, STK-001, and we may forego or delay pursuit of opportunities with other product candidates or for other indications that later prove to have greater commercial potential. Our resource allocation decisions may cause us to fail to capitalize on viable commercial products or profitable market opportunities. Our spending on current and future research and development programs and product candidates for specific indications may not yield any commercially viable products. Our understanding and evaluation of biological targets for the discovery and development of new product candidates may fail to identify challenges encountered in subsequent preclinical and clinical development. If we do not accurately evaluate the commercial potential or target market for a particular product candidate, we may relinquish valuable rights to that product candidate through collaboration, licensing or other royalty arrangements in cases in which it would have been more advantageous for us to retain sole development and commercialization rights.

Any product candidate for which we obtain marketing approval will be subject to extensive post-marketing regulatory requirements and could be subject to post-marketing restrictions or withdrawal from the market, and we may be subject to penalties if we fail to comply with regulatory requirements or if we experience unanticipated problems with our product candidates, when and if any of them are approved.

Our product candidates and the activities associated with their development and potential commercialization, including their testing, manufacturing, recordkeeping, labeling, storage, approval, advertising, promotion, sale and distribution, are subject to comprehensive regulation by the FDA and other U.S. and international regulatory authorities. These requirements include submissions of safety and other post-marketing information and reports, registration and listing requirements, requirements relating to manufacturing, including current Good Manufacturing Practices, or cGMPs, quality control, quality assurance and corresponding maintenance of records and documents, including periodic inspections by the FDA and other regulatory authorities and requirements regarding the distribution of samples to providers and recordkeeping.

The FDA may also impose requirements for costly post-marketing studies or clinical trials and surveillance to monitor the safety or efficacy of any approved product. The FDA closely regulates the post-approval marketing and promotion of drugs and biologics to ensure drugs and biologics are marketed only for the approved indications and in accordance with the provisions of the approved labeling. The FDA imposes stringent restrictions on manufacturers' communications regarding use of their products. If we promote our product candidates in a manner inconsistent with FDA-approved labeling or otherwise not in compliance with FDA regulations, we may be subject to enforcement action. Violations of the Federal Food, Drug, and Cosmetic Act relating to the promotion of prescription drugs may lead to investigations alleging violations of federal and state healthcare fraud and abuse laws, as well as state consumer protection laws and similar laws in international jurisdictions.

In addition, later discovery of previously unknown adverse events or other problems with our product candidates, manufacturers or manufacturing processes, or failure to comply with regulatory requirements, may yield various results, including:

- restrictions on such product candidates, manufacturers or manufacturing processes;
- restrictions on the labeling or marketing of a product;
- restrictions on product distribution or use;
- requirements to conduct post-marketing studies or clinical trials;
- warning or untitled letters;
- withdrawal of any approved product from the market;
- refusal to approve pending applications or supplements to approved applications that we submit;
- recall of product candidates;
- fines, restitution or disgorgement of profits or revenues;
- suspension or withdrawal of marketing approvals;
- refusal to permit the import or export of our product candidates;
- product seizure; or
- injunctions or the imposition of civil or criminal penalties.

Non-compliance with European requirements regarding safety monitoring or pharmacovigilance, and with requirements related to the development of products for the pediatric population, can also result in significant financial penalties. Similarly, failure to comply with Europe's requirements regarding the protection of personal information can also lead to significant penalties and sanctions.

Our failure to obtain regulatory approval in international jurisdictions would prevent us from marketing our product candidates outside the United States.

To market and sell STK-001 and our future product candidates in other jurisdictions, we must obtain separate marketing approvals and comply with numerous and varying regulatory requirements. The approval procedure varies among countries and can involve additional testing. The time required to obtain approval may differ substantially from that required to obtain FDA approval. The regulatory approval process outside the United States generally includes all of the risks associated with obtaining FDA approval. In addition, in many countries outside the United States, we must secure product reimbursement approvals before regulatory authorities will approve the product for sale in that country. Failure to obtain foreign regulatory approvals or non-compliance with foreign regulatory requirements could result in significant delays, difficulties and costs for us and could delay or prevent the introduction of our product candidates in certain countries. The United Kingdom's pending exit from the European Union, or the EU, which is referred to as "Brexit," continues to create political and economic uncertainty, particularly in the United Kingdom and the EU. Since a significant proportion of the regulatory framework in the United Kingdom is derived from EU directives and regulations, the withdrawal of the United Kingdom from the EU could materially impact the regulatory regime with respect to the approval of our product candidates in the United Kingdom or the EU.

If we fail to comply with the regulatory requirements in international markets and receive applicable marketing approvals, our target market will be reduced and our ability to realize the full market potential of our product candidates will be harmed and our business will be adversely affected. We may not obtain foreign regulatory approvals on a timely basis, if at all. Our failure to obtain approval of any of our product candidates by regulatory authorities in another country may significantly diminish the commercial prospects of that product candidate and our business prospects could decline.

STK-001 and our future product candidates may cause undesirable and unforeseen side effects or be perceived by the public as unsafe, which could delay or prevent their advancement into clinical trials or regulatory approval, limit the commercial potential or result in significant negative consequences.

Although other ASOs have received regulatory approval, our method of seeking to upregulate protein expression by targeting the underlying genetic causes of haploinsufficiencies presents a new approach to disease treatment, which means there is uncertainty associated with the safety profile of STK-001 and our future product candidates and drugs in the antisense oligonucleotide class.

In addition to side effects caused by our product candidates, the intrathecal administration process or related procedures also can cause adverse side effects. If any such adverse events occur, our clinical trials could be suspended or terminated. If we are unable to demonstrate that any adverse events were caused by the administration process or related procedures, the FDA, the European Commission, the EMA or other regulatory authorities could order us to cease further development of, or deny approval of, our product candidates for any or all targeted indications. Even if we can demonstrate that all future serious adverse events are not product-related, such occurrences could affect patient recruitment or the ability of enrolled patients to complete the trial. Moreover, if we elect, or are required, to not initiate, delay, suspend or terminate any future clinical trial of any of our product candidates, the commercial prospects of such product candidates may be harmed and our ability to generate product revenues from any of these product candidates may be delayed or eliminated. Any of these occurrences may harm our ability to develop other product candidates, and may adversely affect our

business, financial condition, results of operations and prospects significantly. Finally, SPINRAZA, which is produced by Biogen Inc., is an ASO therapy utilizing intrathecal delivery, and if SPINRAZA is found to cause undesirable side effects or to be unsafe due to a potential class effect, it may adversely affect demand for STK-001 and our other future product candidates. Other ASOs in clinical development utilizing intrathecal delivery could also generate data that could adversely affect the clinical, regulatory or commercial perception of STK-001 and our other future product candidates.

Additionally, if any of our product candidates receives marketing approval, the FDA could require us to adopt a Risk Evaluation and Mitigation Strategy to ensure that the benefits of the product outweigh its risks, which may include, for example, a Medication Guide outlining the risks of the product for distribution to patients and a communication plan to health care practitioners, or other elements to assure safe use of the product. Furthermore, if we or others later identify undesirable side effects caused by our product candidate, several potentially significant negative consequences could result, including:

- regulatory authorities may suspend or withdraw approvals of such product candidate;
- regulatory authorities may require additional warnings on the label;
- we may be required to change the way a product candidate is administered or conduct additional clinical trials;
- we could be sued and held liable for harm caused to patients; and
- our reputation may suffer.

Any of these occurrences may harm our business, financial condition, results of operations and prospects significantly.

A Fast Track Designation by the FDA, even if granted for STK-001 or any of our future product candidates, may not lead to a faster development or regulatory review or approval process, and does not increase the likelihood that our product candidates will receive marketing approval.

We may seek Fast Track Designation for STK-001 or our future product candidates. If a drug is intended for the treatment of a serious or life-threatening condition and the drug demonstrates the potential to address unmet medical needs for this condition, the drug sponsor may apply to the FDA for Fast Track Designation. The FDA has broad discretion whether to grant this designation. Even if we believe a particular product candidate is eligible for this designation, we cannot assure you that the FDA would decide to grant it. Even if we do receive Fast Track Designation, we may not experience a faster development process, review or approval compared to conventional FDA procedures. The FDA may withdraw Fast Track Designation if it believes that the designation is no longer supported by data from our clinical development program. Many drugs that have received Fast Track Designation have failed to obtain approval.

We may also seek accelerated approval for product candidates that have obtained Fast Track Designation. Under the FDA's accelerated approval program, the FDA may approve a drug for a serious or life-threatening illness that provides meaningful therapeutic benefit to patients over existing treatments based upon a surrogate endpoint that is reasonably likely to predict clinical benefit, or on a clinical endpoint that can be measured earlier than irreversible morbidity or mortality, that is reasonably likely to predict an effect on irreversible morbidity or mortality or other clinical benefit, taking into account the severity, rarity or prevalence of the condition and the availability or lack of alternative treatments. For drugs granted accelerated approval, post-marketing confirmatory trials are required to describe the anticipated effect on irreversible morbidity or mortality or other clinical benefit. These confirmatory trials must be completed with due diligence and, in some cases, the FDA may require that the trial be designed and/or initiated prior to approval. Moreover,

the FDA may withdraw approval of any product candidate or indication approved under the accelerated approval pathway if, for example:

- the trial or trials required to verify the predicted clinical benefit of the product candidate fail to verify such benefit or do not demonstrate sufficient clinical benefit to justify the risks associated with the drug;
- other evidence demonstrates that the product candidate is not shown to be safe or effective under the conditions of use;
- we fail to conduct any required post-approval trial of the product candidate with due diligence; or
- we disseminate false or misleading promotional materials relating to the product candidate.

A Breakthrough Therapy Designation by the FDA for STK-001 or our future product candidates may not lead to a faster development or regulatory review or approval process, and it would not increase the likelihood that the product candidate will receive marketing approval.

We may seek a Breakthrough Therapy Designation for STK-001 or one or more of our future product candidates. A breakthrough therapy is defined as a drug that is intended, alone or in combination with one or more other drugs, to treat a serious or life-threatening disease or condition, and preliminary clinical evidence indicates that the drug may demonstrate substantial improvement over existing therapies on one or more clinically significant endpoints, such as substantial treatment effects observed early in clinical development. For drugs that have been designated as breakthrough therapies, interaction and communication between the FDA and the sponsor of the trial can help to identify the most efficient path for clinical development while minimizing the number of patients placed in ineffective control regimens. Drugs designated as breakthrough therapies by the FDA are also eligible for priority review if supported by clinical data at the time of the submission of the NDA.

Designation as a breakthrough therapy is at the discretion of the FDA. Accordingly, even if we believe that one of our product candidates meets the criteria for designation as a breakthrough therapy, the FDA may disagree and instead determine not to make such designation. In any event, the receipt of a Breakthrough Therapy Designation for a drug may not result in a faster development process, review, or approval compared to drugs considered for approval under conventional FDA procedures and it would not assure ultimate approval by the FDA. In addition, even if one or more of our product candidates qualify as breakthrough therapies, the FDA may later decide that the product candidate no longer meets the conditions for qualification or it may decide that the time period for FDA review or approval will not be shortened.

Enacted and future legislation may increase the difficulty and cost for us to obtain marketing approval of and commercialize our product candidates and may affect the prices we may set.

Existing regulatory policies may change and additional government regulations may be enacted that could prevent, limit or delay regulatory approval of our product candidates. We cannot predict the likelihood, nature or extent of government regulation that may arise from future legislation or administrative action, either in the United States or abroad. If we are slow or unable to adapt to changes in existing requirements or the adoption of new requirements or policies, or if we are not able to maintain regulatory compliance, we may lose any marketing approval that we may have obtained and we may not achieve or sustain profitability.

For example, in March 2010, the Patient Protection and Affordable Care Act, as amended by the Health Care and Education Reconciliation Act of 2010, or collectively the Affordable Care Act, or the ACA, was enacted to broaden access to health insurance, reduce or constrain the growth of healthcare spending, enhance remedies against fraud and abuse, add new transparency requirements for health care and health insurance industries, impose new taxes and fees on the health industry and impose additional health policy reforms. As implementation of the ACA is ongoing, the law appears likely to continue the downward pressure on

pharmaceutical pricing, especially under the Medicare program, and may also increase our regulatory burdens and operating costs. The current U.S. presidential administration and U.S. Congress have sought, and we expect they will continue to, seek to modify, repeal, or otherwise invalidate all, or certain provisions of, the ACA. Since January 2017, the current U.S. presidential administration has issued two executive orders and other directives designed to delay the implementation of certain provisions of the ACA or otherwise circumvent some of the requirements for health insurance mandated by the ACA. For example, on October 12, 2017, the current U.S. presidential administration issued an executive order that expands the use of association health plans and allows anyone to purchase short-term health plans that provide temporary, limited insurance. This executive order also calls for the halt of federal payments to health insurers for cost-sharing reductions previously available to lower-income Americans to afford coverage. There is uncertainty with respect to which legislation, if any, will be enacted and the impact the current U.S. presidential administration may have, if any, and any changes likely will take time to unfold, and could have an impact on coverage and reimbursement for healthcare items and services covered by plans that were authorized by the ACA. However, we cannot predict the ultimate content, timing or effect of any healthcare reform legislation or the impact of potential legislation on us. In addition, other legislative changes have been proposed and adopted since the ACA was enacted. These changes included aggregate reductions to Medicare payments to providers of up to 2% per fiscal year, effective April 1, 2013, which, due to subsequent legislative amendments, will stay in effect through 2027 unless additional Congressional action is taken. In January 2013, the American Taxpayer Relief Act of 2012 was signed into law, which, among other things, reduced Medicare payments to several providers, and increased the statute of limitations period for the government to recover overpayments to providers from three to five years. These new laws may result in additional reductions in Medicare and other healthcare funding, which could have a material adverse effect on customers for our drugs, if approved, and accordingly, our financial operations. Additionally, on May 30, 2018, the Trickett Wendler, Frank Mongiello, Jordan McLinn, and Matthew Bellina Right to Try Act of 2017 was signed into law. The law, among other things, provides a federal framework for certain patients to access certain investigational new drug products that have completed a Phase I clinical trial and that are undergoing investigation for FDA approval. Under certain circumstances, eligible patients can seek treatment without enrolling in clinical trials and without obtaining FDA authorization under an FDA expanded access program; however, manufacturers are not obligated to provide investigational new drug products under the current federal right to try law. We may choose to seek an expanded access program for our product candidates, or to utilize comparable rules in other countries that allow the use of a drug, on a named patient basis or under a compassionate use program.

We expect that the ACA, as well as other healthcare reform measures that may be adopted in the future, may result in more rigorous coverage criteria and in additional downward pressure on the price that we receive for any approved product. Any reduction in reimbursement from Medicare or other government programs may result in a similar reduction in payments from private payors. The implementation of cost containment measures or other healthcare reforms may prevent us from being able to generate revenue, attain profitability, or commercialize our product candidates.

Legislative and regulatory proposals have been made to expand post-approval requirements and restrict sales and promotional activities for pharmaceutical products. We cannot be sure whether additional legislative changes will be enacted, or whether FDA regulations, guidance or interpretations will be changed, or what the impact of such changes on the marketing approvals of our product candidates, if any, may be. In addition, increased scrutiny by the U.S. Congress of the FDA’s approval process may significantly delay or prevent marketing approval, as well as subject us to more stringent product labeling and post-marketing testing and other requirements.

We may be unsuccessful in obtaining Orphan Drug Designation or transfer of designations obtained by others for future product candidates. and, even if we obtain such designation, we may be unable to maintain the benefits associated with Orphan Drug Designation, including the potential for market exclusivity, for STK-001 or our future product candidates.

Regulatory authorities in some jurisdictions, including the United States and Europe, may designate drugs intended to treat relatively small patient populations as orphan drugs. Under the Orphan Drug Act, the FDA may designate a drug as an orphan drug if it is intended to treat a rare disease or condition, which is defined as a patient population of fewer than 200,000 individuals in the United States. In the United States, Orphan Drug Designation entitles a party to financial incentives such as opportunities for tax credits for qualified clinical research costs and exemption from prescription drug user fees. Similarly, in the EU, the European Commission grants Orphan Drug Designation after receiving the opinion of the EMA's Committee for Orphan Medicinal Products on an Orphan Drug Designation application. In the EU, Orphan Drug Designation is intended to promote the development of drug that are intended for the diagnosis, prevention or treatment of life-threatening or chronically debilitating conditions affecting not more than five in 10,000 persons in the EU and for which no satisfactory method of diagnosis, prevention or treatment has been authorized (or the product would be a significant benefit to those affected). In the EU, Orphan Drug Designation entitles a party to financial incentives such as reduction of fees or fee waivers.

Generally, if a drug with an Orphan Drug Designation subsequently receives the first marketing approval for the indication for which it has such designation, the drug is entitled to a period of marketing exclusivity, which precludes EMA or the FDA from approving another marketing application for the same drug and indication for that time period, except in limited circumstances. If a competitor is able to obtain orphan drug exclusivity prior to us for a product that constitutes the same active moiety and treats the same indications as our product candidates, we may not be able to obtain approval of our drug by the applicable regulatory authority for a significant period of time unless we are able to show that our drug is clinically superior to the approved drug. The applicable period is seven years in the United States and ten years in the EU. The EU exclusivity period can be reduced to six years if a drug no longer meets the criteria for Orphan Drug Designation or if the drug is sufficiently profitable so that market exclusivity is no longer justified.

As part of our business strategy, we have applied for Orphan Drug Designation for STK-001 in the United States, and we may seek such designation in Europe and other countries. However, Orphan Drug Designation does not guarantee future orphan drug marketing exclusivity.

Even after an orphan drug is approved, the FDA can also subsequently approve a later application for the same drug for the same condition if the FDA concludes that the later drug is clinically superior in that it is shown to be safer in a substantial portion of the target populations, more effective or makes a major contribution to patient care. In addition, a designated orphan drug may not receive orphan drug exclusivity if it is approved for a use that is broader than the indication for which it received orphan designation. Moreover, orphan drug exclusive marketing rights in the United States may be lost if the FDA later determines that the request for designation was materially defective or if we are unable to manufacture sufficient quantities of the product to meet the needs of patients with the rare disease or condition. Orphan Drug Designation neither shortens the development time or regulatory review time of a drug nor gives the drug any advantage in the regulatory review or approval process.

A Rare Pediatric Disease designation by the FDA does not guarantee that the NDA for the product will qualify for a priority review voucher upon approval, and it does not lead to a faster development or regulatory review process, or increase the likelihood that STK-001 or any of our future product candidates will receive marketing approval.

Under the Rare Pediatric Disease Priority Review Voucher program, upon the approval of a qualifying NDA for the treatment of a rare pediatric disease, the sponsor of such an application would be eligible for a rare pediatric disease priority review voucher that can be used to obtain priority review for a subsequent Biologics License Application, or BLA, or NDA. We may seek Rare Pediatric Disease designations for STK-001. If a product candidate is designated before October 1, 2020, it is eligible to receive a voucher if it is approved before October 1, 2022. However, there is no expectation that STK-001 or any of our future product candidates will be approved by that date, or at all, and, therefore, we may not be in a position to obtain priority review vouchers prior to expiration of the program, unless Congress further reauthorizes the program. Additionally, designation of a drug for a rare pediatric disease does not guarantee that an NDA will meet the eligibility criteria for a rare pediatric disease priority review voucher at the time the application is approved. Finally, a Rare Pediatric Disease Designation does not lead to faster development or regulatory review of the product, or increase the likelihood that it will receive marketing approval.

The FDA's ability to review and approve new products may be hindered by a variety of factors, including budget and funding levels, ability to hire and retain key personnel, and statutory, regulatory and policy changes.

The ability of the FDA to review and approve new products can be affected by a variety of factors, including budget and funding levels, ability to hire and retain key personnel, and statutory, regulatory, and policy changes. In addition, government funding of other government agencies that fund research and development activities is subject to the political process, which is inherently fluid and unpredictable.

The ability of the FDA and other government agencies to properly administer their functions is highly dependent on the levels of government funding and the ability to fill key leadership appointments, among various factors. Currently, the FDA Commissioner position is vacant, pending the appointment of a new Commissioner by the new presidential administration. The confirmation process for a new commissioner may not occur efficiently. Delays in filling or replacing key positions could significantly impact the ability of the FDA and other agencies to fulfill their functions, and could greatly impact healthcare and the pharmaceutical industry.

In December 2016, the 21st Century Cures Act was signed into law, and was designed to advance medical innovation and empower the FDA with the authority to directly hire positions related to drug and device development and review. In the past, the FDA was often unable to offer key leadership candidates (including scientists) competitive compensation packages as compared to those offered by private industry. The 21st Century Cures Act is designed to streamline the agency's hiring process and enable the FDA to compete for leadership talent by expanding the narrow ranges that are provided in the existing compensation structures.

Disruptions at the FDA and other governmental agencies may also slow the time necessary for new drugs to be reviewed or approved by necessary government agencies, which would adversely affect our operating results and business.

Our operations and relationships with future customers, providers and third-party payors will be subject to applicable anti-kickback, fraud and abuse and other healthcare laws and regulations, which could expose us to penalties including criminal sanctions, civil penalties, contractual damages, reputational harm and diminished profits and future earnings.

Healthcare providers and third-party payors will play a primary role in the recommendation and prescription of any product candidates for which we obtain marketing approval. Our future arrangements with providers,

third-party payors and customers will subject us to broadly applicable fraud and abuse and other healthcare laws and regulations that may constrain the business or financial arrangements and relationships through which we market, sell and distribute any product candidates for which we obtain marketing approval.

Restrictions under applicable U.S. federal and state healthcare laws and regulations include the following:

- the federal Anti-Kickback Statute prohibits, among other things, persons and entities from knowingly and willfully soliciting, offering, receiving or providing remuneration, directly or indirectly, in cash or in kind, to induce or reward either the referral of an individual for, or the purchase, order or recommendation of, any good or service, for which payment may be made under federal healthcare programs such as Medicare and Medicaid;
- federal false claims laws, including the federal False Claims Act, imposes criminal and civil penalties, including through civil whistleblower or qui tam actions, against individuals or entities for knowingly presenting, or causing to be presented, to the federal government, claims for payment that are false or fraudulent or making a false statement to avoid, decrease or conceal an obligation to pay money to the federal government;
- the federal Health Insurance Portability and Accountability Act of 1996, or HIPAA, imposes criminal and civil liability for, among other things, knowingly and willfully executing or attempting to execute a scheme to defraud any healthcare benefit program or making false statements relating to healthcare matters;
- HIPAA, as amended by the Health Information Technology for Economic and Clinical Health Act, or HITECH, and its implementing regulations, also imposes obligations, including mandatory contractual terms, on certain types of people and entities with respect to safeguarding the privacy, security and transmission of individually identifiable health information;
- the federal Physician Payment Sunshine Act requires applicable manufacturers of covered drugs, devices, biologics, and medical supplies for which payment is available under Medicare, Medicaid, or the Children's Health Insurance Program, with specific exceptions, to report payments and other transfers of value to physicians and teaching hospitals, as well as certain ownership and investment interests held by physicians and their immediate family, which includes annual data collection and reporting obligations; and
- analogous state and foreign laws and regulations, such as state anti-kickback and false claims laws, may apply to sales or marketing arrangements and claims involving healthcare items or services reimbursed by non-governmental third-party payors, including private insurers.

Some state laws require pharmaceutical companies to comply with the pharmaceutical industry's voluntary compliance guidelines and the relevant compliance guidance promulgated by the federal government and may require drug manufacturers to report information related to payments and other transfers of value to physicians and other healthcare providers or marketing expenditures. State and foreign laws also govern the privacy and security of health information in some circumstances, many of which differ from each other in significant ways and often are not preempted by HIPAA, thus complicating compliance efforts.

Efforts to ensure that our business arrangements with third parties will comply with applicable healthcare laws and regulations will involve substantial costs. It is possible that governmental authorities will conclude that our business practices may not comply with current or future statutes, regulations or case law involving applicable fraud and abuse or other healthcare laws and regulations. If our operations are found to be in violation of any of these laws or any other governmental regulations that may apply to us, we may be subject to significant civil, criminal and administrative penalties, damages, fines, imprisonment, exclusion of product candidates from government-funded healthcare programs, such as Medicare and Medicaid, disgorgement, contractual damages,

reputational harm, diminished profits and future earnings, and the curtailment or restructuring of our operations. If any of the physicians or other healthcare providers or entities with whom we expect to do business is found to be not in compliance with applicable laws, they may be subject to criminal, civil or administrative sanctions, including exclusions from government-funded healthcare programs.

Risks related to commercialization and manufacturing

The commercial success of our product candidates, including STK-001, will depend upon their degree of market acceptance by providers, patients, patient advocacy groups, third-party payors and the general medical community.

Ethical, social and legal concerns about genetic treatments generally could result in additional regulations restricting or prohibiting our product candidates. Even with the requisite approvals from the FDA, the EMA and other regulatory authorities internationally, the commercial success of our product candidates will depend, in part, on the acceptance of providers, patients and third-party payors of drugs designed to increase protein expression in general, and our product candidates in particular, as medically necessary, cost-effective and safe. In addition, we may face challenges in seeking to establish and grow sales of STK-001, including acceptance of the lumbar puncture and intrathecal administration, which carries risks of infection or other complications. Any product that we commercialize may not gain acceptance by providers, patients, patient advocacy groups, third-party payors and the general medical community. If these products do not achieve an adequate level of acceptance, we may not generate significant product revenue and may not become profitable. The degree of market acceptance of genetic medicines and, in particular, STK-001 and our future product candidates, if approved for commercial sale, will depend on several factors, including:

- the efficacy, durability and safety of such product candidates as demonstrated in clinical trials;
- the potential and perceived advantages of product candidates over alternative treatments;
- the cost of treatment relative to alternative treatments;
- the clinical indications for which the product candidate is approved by the FDA or the European Commission;
- the willingness of providers to prescribe new therapies;
- the willingness of the target patient population to try new therapies;
- the prevalence and severity of any side effects;
- product labeling or product insert requirements of the FDA, EMA or other regulatory authorities, including any limitations or warnings contained in a product's approved labeling;
- the willingness of providers to prescribe, and of patients to receive, intrathecal injections;
- the strength of marketing and distribution support;
- the timing of market introduction of competitive products;
- the quality of our relationships with patient advocacy groups;
- publicity concerning our product candidates or competing products and treatments; and
- sufficient third-party payor coverage and adequate reimbursement.

Even if a potential product displays a favorable efficacy and safety profile in preclinical studies and clinical trials, market acceptance of the product will not be fully known until after it is launched.

The pricing, insurance coverage and reimbursement status of newly approved products is uncertain. Failure to obtain or maintain adequate coverage and reimbursement for our product candidates, if approved, could limit our ability to market those products and decrease our ability to generate product revenue.

Our target indications, including Dravet syndrome, are indications with small patient populations. For product candidates that are designed to treat smaller patient populations to be commercially viable, the reimbursement for such product candidates must be higher, on a relative basis, to account for the lack of volume. Accordingly, we will need to implement a coverage and reimbursement strategy for any approved product candidate that accounts for the smaller potential market size. If we are unable to establish or sustain coverage and adequate reimbursement for any future product candidates from third-party payors, the adoption of those product candidates and sales revenue will be adversely affected, which, in turn, could adversely affect the ability to market or sell those product candidates, if approved.

We expect that coverage and reimbursement by third-party payors will be essential for most patients to be able to afford these treatments. Accordingly, sales of STK-001 and our future product candidates will depend substantially, both domestically and internationally, on the extent to which the costs of our product candidates will be paid by health maintenance, managed care, pharmacy benefit and similar healthcare management organizations, or will be reimbursed by government authorities, private health coverage insurers and other third-party payors. Even if coverage is provided, the approved reimbursement amount may not be high enough to allow us to establish or maintain pricing sufficient to realize a sufficient return on our investment.

There is significant uncertainty related to the insurance coverage and reimbursement of newly approved products. In the United States, the principal decisions about reimbursement by government authorities for new products are typically made by the Centers for Medicare & Medicaid Services, or CMS, since CMS decides whether and to what extent a new product will be covered and reimbursed under Medicare. Private payors tend to follow CMS to a substantial degree. However, one payor’s determination to provide coverage for a drug product does not assure that other payors will also provide coverage for the drug product. Further, a payor’s decision to provide coverage for a drug product does not imply that an adequate reimbursement rate will be approved. Reimbursement agencies in Europe may be more conservative than CMS. For example, a number of cancer drugs have been approved for reimbursement in the United States and have not been approved for reimbursement in certain European countries

Outside the United States, international operations are generally subject to extensive governmental price controls and other market regulations, and we believe the increasing emphasis on cost-containment initiatives in Europe, Canada and other countries has and will continue to put pressure on the pricing and usage of therapeutics such as our product candidates. In many countries, particularly the countries of the EU, the prices of medical products are subject to varying price control mechanisms as part of national health systems. In these countries, pricing negotiations with governmental authorities can take considerable time after the receipt of marketing approval for a product. To obtain reimbursement or pricing approval in some countries, we may be required to conduct a clinical trial that compares the cost-effectiveness of our product candidate to other available therapies. In general, the prices of products under such systems are substantially lower than in the United States. Other countries allow companies to fix their own prices for products, but monitor and control company profits. Additional foreign price controls or other changes in pricing regulation could restrict the amount that we are able to charge for our product candidates. Accordingly, in markets outside the United States, the reimbursement for our product candidates may be reduced compared with the United States and may be insufficient to generate commercially reasonable revenues and profits.

Moreover, increasing efforts by governmental and third-party payors, in the United States and internationally, to cap or reduce healthcare costs may cause such organizations to limit both coverage and level of reimbursement for new products approved and, as a result, they may not cover or provide adequate payment

for our product candidates. We expect to experience pricing pressures in connection with the sale of any of our product candidates due to the trend toward managed healthcare, the increasing influence of certain third-party payors, such as health maintenance organizations, and additional legislative changes. The downward pressure on healthcare costs in general, particularly prescription drugs and surgical procedures and other treatments, has become very intense. As a result, increasingly high barriers are being erected to the entry of new products into the healthcare market. Recently there have been instances in which third-party payors have refused to reimburse treatments for patients for whom the treatment is indicated in the FDA-approved product label. Even if we are successful in obtaining FDA approvals to commercialize our product candidates, we cannot guarantee that we will be able to secure reimbursement for all patients for whom treatment with our product candidates is indicated.

In addition to CMS and private payors, professional organizations such as the American Medical Association, or the AMA, can influence decisions about reimbursement for new products by determining standards for care. In addition, many private payors contract with commercial vendors who sell software that provide guidelines that attempt to limit utilization of, and therefore reimbursement for, certain products deemed to provide limited benefit to existing alternatives. Such organizations may set guidelines that limit reimbursement or utilization of our product candidates. Even if favorable coverage and reimbursement status is attained for one or more product candidates for which we or our collaborators receive regulatory approval, less favorable coverage policies and reimbursement rates may be implemented in the future.

If third parties on which we depend to conduct our planned preclinical studies, or any future clinical trials, do not perform as contractually required, fail to satisfy regulatory or legal requirements or miss expected deadlines, our development program could be delayed with adverse effects on our business, financial condition, results of operations and prospects.

We rely on third parties for genetic testing, and on third party contract research organizations, or CROs, contract manufacturing organizations, or CMOs, consultants and others to design, conduct, supervise and monitor key activities relating to, discovery, manufacturing, preclinical studies and clinical trials of our product candidates, and we intend to do the same for future activities relating to existing and future programs. Because we rely on third parties and do not have the ability to conduct all required testing, discovery, manufacturing, preclinical studies or clinical trials independently, we have less control over the timing, quality and other aspects of discovery, manufacturing, preclinical studies and clinical trials than we would if we conducted them on our own. These investigators, CROs, CMOs and consultants are not our employees and we have limited control over the amount of time and resources that they dedicate to our programs. These third parties may have contractual relationships with other entities, some of which may be our competitors, which may draw time and resources from our programs. The third parties we contract with might not be diligent, careful or timely in conducting our discovery, manufacturing, preclinical studies or clinical trials, resulting in testing, discovery, manufacturing, preclinical studies or clinical trials being delayed or unsuccessful, in whole or in part.

If we cannot contract with acceptable third parties on commercially reasonable terms, or at all, or if these third parties do not carry out their contractual duties, satisfy legal and regulatory requirements for the conduct of preclinical studies or clinical trials or meet expected deadlines, our clinical development programs could be delayed and otherwise adversely affected. In all events, we are responsible for ensuring that each of our preclinical studies and clinical trials is conducted in accordance with the general investigational plan and protocols for the trial. Our reliance on third parties that we do not control does not relieve us of these responsibilities and requirements. Any such event could have an adverse effect on our business, financial condition, results of operations and prospects.

We face significant competition in an environment of rapid technological change and it is possible that our competitors may achieve regulatory approval before us or develop therapies that are more advanced or effective than ours, which may harm our business, financial condition and our ability to successfully market or commercialize STK-001 and our future product candidates.

The biotechnology and pharmaceutical industries, including the genetic medicine and antisense oligonucleotide fields, are characterized by rapidly changing technologies, competition and a strong emphasis on intellectual property. We are aware of several companies focused on developing ASO treatments in various indications as well as several companies addressing other methods for modifying genes and regulating protein expression. We may also face competition from large and specialty pharmaceutical and biotechnology companies, academic research institutions, government agencies and public and private research institutions.

Although few companies focus treatments on Dravet syndrome, such as Encoded Therapeutics, Inc., numerous treatments for epilepsy exist, including cannabidiols, such as GW Pharmaceuticals, plc's Epidiolex, GABA receptor agonists, such as clobazam, and glutamate blockers, such as topiramate. In addition, numerous compounds are in clinical development for treatment of epilepsy. We believe the clinical development pipeline includes cannabinoids, 5-HT release stimulants, cholesterol 24-hydroxylase inhibitors, and sodium channel antagonists from a variety of companies. In addition to competition from these small molecule drugs, any products we may develop may also face competition from other types of therapies, such as gene therapy, gene editing, modified mRNA therapies or other ASO approaches.

Many of our potential competitors, alone or with their strategic partners, have substantially greater financial, technical and other resources than we do, such as larger research and development, clinical, marketing and manufacturing organizations. Mergers and acquisitions in the biotechnology and pharmaceutical industries may result in even more resources being concentrated among a smaller number of competitors. Our commercial opportunity could be reduced or eliminated if competitors develop and commercialize products that are safer, more effective, have fewer or less severe side effects, are more convenient or are less expensive than any product candidates that we may develop. Competitors also may obtain FDA or other regulatory approval for their products more rapidly than we may obtain approval for ours, which could result in our competitors establishing a strong market position before we are able to enter the market, if ever. Additionally, new or advanced technologies developed by our competitors may render our current or future product candidates uneconomical or obsolete, and we may not be successful in marketing our product candidates against competitors.

To become and remain profitable, we must develop and eventually commercialize product candidates with significant market potential, which will require us to be successful in a range of challenging activities. These activities include, among other things, completing preclinical studies and initiating and completing clinical trials of our product candidates, obtaining marketing approval for these product candidates, manufacturing, marketing and selling those products that are approved and satisfying any post marketing requirements. We may never succeed in any or all of these activities and, even if we do, we may never generate revenues that are significant or large enough to achieve profitability. If we do achieve profitability, we may not be able to sustain or increase profitability on a quarterly or annual basis. Our failure to become and remain profitable would decrease the value of our company and could impair our ability to raise capital, maintain our research and development efforts, expand our business or continue our operations. A decline in the value of our company also could cause you to lose all or part of your investment.

The manufacture of drugs is complex and our third-party manufacturers may encounter difficulties in production. If any of our third-party manufacturers encounter such difficulties, our ability to provide supply of STK-001 or our future product candidates for clinical trials, our ability to obtain marketing approval, or our ability to provide supply of our product candidates for patients, if approved, could be delayed or stopped.

We have established manufacturing relationships with a limited number of suppliers to manufacture raw materials and the drug substance of any product candidate for which we are responsible for preclinical or clinical development. Each supplier may require licenses to manufacture such components if such processes are not owned by the supplier or in the public domain. As part of any marketing approval, a manufacturer and its processes are required to be qualified by the FDA prior to commercialization. If supply from the approved vendor is interrupted, there could be a significant disruption in commercial supply. An alternative vendor would need to be qualified through an NDA supplement which could result in further delay. The FDA or other regulatory agencies outside of the United States may also require additional studies if a new supplier is relied upon for commercial production. Switching vendors may involve substantial costs and is likely to result in a delay in our desired clinical and commercial timelines.

The process of manufacturing drugs is complex, highly-regulated and subject to multiple risks. Manufacturing drugs is highly susceptible to product loss due to contamination, equipment failure, improper installation or operation of equipment, vendor or operator error, inconsistency in yields, variability in product characteristics and difficulties in scaling the production process. Even minor deviations from normal manufacturing processes could result in reduced production yields, product defects and other supply disruptions. If microbial, viral or other contaminations are discovered at the facilities of our manufacturers, such facilities may need to be closed for an extended period of time to investigate and remedy the contamination, which could delay clinical trials and adversely harm our business. Moreover, if the FDA determines that our manufacturers are not in compliance with FDA laws and regulations, including those governing cGMPs, the FDA may deny NDA approval until the deficiencies are corrected or we replace the manufacturer in our NDA with a manufacturer that is in compliance.

In addition, there are risks associated with large scale manufacturing for clinical trials or commercial scale including, among others, cost overruns, potential problems with process scale-up, process reproducibility, stability issues, compliance with good manufacturing practices, lot consistency and timely availability of raw materials. Even if we or our collaborators obtain regulatory approval for any of our product candidates, there is no assurance that manufacturers will be able to manufacture the approved product to specifications acceptable to the FDA or other regulatory authorities, to produce it in sufficient quantities to meet the requirements for the potential launch of the product or to meet potential future demand. If our manufacturers are unable to produce sufficient quantities for clinical trials or for commercialization, commercialization efforts would be impaired, which would have an adverse effect on our business, financial condition, results of operations and prospects.

Our reliance on a limited number of manufacturers, the complexity of drug manufacturing and the difficulty of scaling up a manufacturing process could cause the delay of clinical trials, regulatory submissions, required approvals or commercialization of our product candidates, cause us to incur higher costs and prevent us from commercializing our product candidates successfully. Furthermore, if our suppliers fail to deliver the required commercial quantities of materials on a timely basis and at commercially reasonable prices, and we are unable to secure one or more replacement suppliers capable of production in a timely manner at a substantially equivalent cost, our clinical trials may be delayed or we could lose potential revenue.

If we are unable to establish sales and marketing capabilities or enter into agreements with third parties to market and sell STK-001 and our future product candidates, we may be unable to generate any revenues.

We currently do not have an organization for the sales, marketing and distribution of STK-001 and our future product candidates and the cost of establishing and maintaining such an organization may exceed the cost-effectiveness of doing so. To market any products that may be approved, we must build our sales, marketing, managerial and other non-technical capabilities or make arrangements with third parties to perform these services. With respect to certain of our current programs as well as future programs, we may rely completely on an alliance partner for sales and marketing. In addition, although we intend to establish a sales organization if we are able to obtain approval to market any product candidates, we may enter into strategic alliances with third parties to develop and commercialize STK-001 and other future product candidates, including in markets outside of the United States or for other large markets that are beyond our resources. This will reduce the revenue generated from the sales of these products.

Any future strategic alliance partners may not dedicate sufficient resources to the commercialization of our product candidates or may otherwise fail in their commercialization due to factors beyond our control. If we are unable to establish effective alliances to enable the sale of our product candidates to healthcare professionals and in geographical regions, including the United States, that will not be covered by our own marketing and sales force, or if our potential future strategic alliance partners do not successfully commercialize the product candidates, our ability to generate revenues from product sales will be adversely affected.

If we are unable to establish adequate sales, marketing and distribution capabilities, whether independently or with third parties, we may not be able to generate sufficient product revenue and may not become profitable. We will be competing with many companies that currently have extensive and well-funded marketing and sales operations. Without an internal team or the support of a third party to perform marketing and sales functions, we may be unable to compete successfully against these more established companies.

We may expend our limited resources to pursue a particular product candidate or indication and fail to capitalize on product candidates or indications that may be more profitable or for which there is a greater likelihood of success.

Because we have limited financial and managerial resources, we focus on research programs and product candidates that we identify for specific indications. As a result, we may forego or delay pursuit of opportunities with other product candidates or for other indications that later prove to have greater commercial potential. Our resource allocation decisions may cause us to fail to timely capitalize on viable commercial products or profitable market opportunities. Our spending on current and future research and development programs and product candidates for specific indications may not yield any commercially viable products. If we do not accurately evaluate the commercial potential or target market for a particular product candidate, we may relinquish valuable rights to that product candidate through collaboration, licensing or other royalty arrangements in cases in which it would have been more advantageous for us to retain sole development and commercialization rights to such product candidate.

We may not be successful in finding strategic collaborators for continuing development of certain of our future product candidates or successfully commercializing or competing in the market for certain indications.

In the future, we may decide to collaborate with non-profit organizations, universities, pharmaceutical and biotechnology companies for the development and potential commercialization of existing and new product candidates. We face significant competition in seeking appropriate collaborators. Whether we reach a definitive agreement for a collaboration will depend, among other things, upon our assessment of the collaborator’s resources and expertise, the terms and conditions of the proposed collaboration and the proposed collaborator’s evaluation of a number of factors. Those factors may include the design or results of clinical

trials, the likelihood of approval by the FDA or similar regulatory authorities outside the United States, the potential market for the subject product candidate, the costs and complexities of manufacturing and delivering such product candidate to patients, the potential of competing drugs, the existence of uncertainty with respect to our ownership of technology, which can exist if there is a challenge to such ownership without regard to the merits of the challenge and industry and market conditions generally. The collaborator may also consider alternative product candidates or technologies for similar indications that may be available to collaborate on and whether such a collaboration could be more attractive than the one with us for our product candidate. The terms of any additional collaborations or other arrangements that we may establish may not be favorable to us. Collaborations are complex and time-consuming to negotiate and document. In addition, there have been a significant number of recent business combinations among large pharmaceutical companies that have resulted in a reduced number of potential future collaborators.

We may not be able to negotiate collaborations on a timely basis, on acceptable terms, or at all. If we are unable to do so, we may have to curtail the development of the product candidate for which we are seeking to collaborate, reduce or delay its development program or one or more of our other development programs, delay its potential commercialization or reduce the scope of any sales or marketing activities, or increase our expenditures and undertake development or commercialization activities at our own expense. If we elect to increase our expenditures to fund development or commercialization activities on our own, we may need to obtain additional capital, which may not be available to us on acceptable terms or at all. If we do not have sufficient funds, we may not be able to further develop our product candidates or bring them to market and generate product revenue.

The success of any potential collaboration arrangements will depend heavily on the efforts and activities of our collaborators. Collaborators generally have significant discretion in determining the efforts and resources that they will apply to these collaborations. Disagreements between parties to a collaboration arrangement regarding clinical development and commercialization matters can lead to delays in the development process or commercializing the applicable product candidate and, in some cases, termination of such collaboration arrangements. These disagreements can be difficult to resolve if neither of the parties has final decision-making authority. Collaborations with pharmaceutical or biotechnology companies and other third parties often are terminated or allowed to expire by the other party. Any such termination or expiration would adversely affect us financially and could harm our business reputation.

Risks related to our financial position

We have a history of operating losses, and we may not achieve or sustain profitability. We anticipate that we will continue to incur losses for the foreseeable future. If we fail to obtain additional funding to conduct our planned research and development effort, we could be forced to delay, reduce or eliminate our product development programs or commercial development efforts.

We are an early-stage biotechnology company with a limited operating history on which to base your investment decision. Biotechnology product development is a highly speculative undertaking and involves a substantial degree of risk. Our operations to date have been limited primarily to organizing and staffing our company, business planning, raising capital, acquiring and developing product and technology rights, manufacturing, and conducting research and development activities for our product candidates. We have never generated any revenue from product sales. We have not obtained regulatory approvals for any of our product candidates, and have funded our operations to date through proceeds from sales of our preferred stock and common stock.

We have incurred net losses in each year since our inception. We incurred a net loss of $12.5 million and $5.6 million for the years ended December 31, 2018 and 2017, respectively, and for the three months ended March 31, 2019 and 2018 we incurred a net loss of $5.7 million and $1.9 million, respectively. As of December 31,

2018 and March 31, 2019, we had an accumulated deficit of $25.7 million and $31.5 million, respectively. Substantially all of our operating losses have resulted from costs incurred in connection with our research and development programs and from general and administrative costs associated with our operations. We expect to continue to incur significant expenses and operating losses over the next several years and for the foreseeable future as we intend to continue to conduct research and development, clinical testing, regulatory compliance activities, manufacturing activities, and, if any of our product candidates is approved, sales and marketing activities that, together with anticipated general and administrative expenses, will likely result in us incurring significant losses for the foreseeable future. Our prior losses, combined with expected future losses, have had and will continue to have an adverse effect on our stockholders' equity and working capital.

We expect that we will need to raise additional funding before we can expect to become profitable from any potential future sales of STK-001 or our future product candidates. This additional financing may not be available on acceptable terms or at all. Failure to obtain this necessary capital when needed may force us to delay, limit or terminate our product development efforts or other operations.

We will require substantial future capital in order to complete planned and future preclinical and clinical development for STK-001 and other future product candidates, if any, and potentially commercialize these product candidates. Based upon our current operating plan, we believe that the net proceeds from this offering, together with our existing cash, cash equivalents and restricted cash as of March 31, 2019, will enable us to fund our operating expenses and capital expenditure requirements through the end of 2022. We expect our spending levels to increase in connection with our preclinical studies and clinical trials of our product candidates. In addition, if we obtain marketing approval for any of our product candidates, we expect to incur significant expenses related to commercial launch, product sales, medical affairs, marketing, manufacturing and distribution. Furthermore, we expect to incur additional costs associated with operating as a public company. Accordingly, we will need to obtain substantial additional funding in connection with our continuing operations. If we are unable to raise capital when needed or on attractive terms, we would be forced to delay, reduce or eliminate certain of our licensing activities, our research and development programs or other operations.

Additional capital might not be available when we need it and our actual cash requirements might be greater than anticipated. If we require additional capital at a time when investment in our industry or in the marketplace in general is limited, we might not be able to raise funding on favorable terms if at all. If we are not able to obtain financing on terms favorable to us, we may need to cease or reduce development or commercialization activities, sell some or all of our assets or merge with another entity, which could result in a loss of all or part of your investment.

Our operations have consumed significant amounts of cash since inception. As of March 31, 2019, our cash, cash equivalents and restricted cash were $98.9 million.

Our future capital requirements will depend on many factors, including:

- the costs associated with the scope, progress and results of discovery, preclinical development, laboratory testing and clinical trials for our product candidates;
- the costs associated with the development of our internal manufacturing facility and processes;
- the costs related to the extent to which we enter into partnerships or other arrangements with third parties to further develop our product candidates;
- the costs and fees associated with the discovery, acquisition or in-license of product candidates or technologies;
- our ability to establish collaborations on favorable terms, if at all;

- the costs of future commercialization activities, if any, including product sales, marketing, manufacturing and distribution, for any of our product candidates for which we receive marketing approval;
- revenue, if any, received from commercial sales of our product candidates, should any of our product candidates receive marketing approval; and
- the costs of preparing, filing and prosecuting patent applications, maintaining and enforcing our intellectual property rights and defending intellectual property-related claims.

Our product candidates, if approved, may not achieve commercial success. Our commercial revenues, if any, will be derived from sales of product candidates that we do not expect to be commercially available for many years, if at all. Accordingly, we will need to continue to rely on additional financing to achieve our business objectives, which may not be available to us on acceptable terms, or at all.

Our limited operating history may make it difficult for you to evaluate the success of our business to date and to assess our future viability.

We are an early-stage biotechnology company formed in June 2014. Our operations to date have been limited to organizing and staffing our company, business planning, raising capital, acquiring our technology, identifying potential product candidates, undertaking research and preclinical studies of our product candidates, manufacturing, and establishing licensing arrangements. We have not yet demonstrated the ability to complete clinical trials of our product candidates, obtain marketing approvals, manufacture a commercial scale product or conduct sales and marketing activities necessary for successful commercialization. Consequently, any predictions you make about our future success or viability may not be as accurate as they could be if we had a longer operating history.

In addition, as a new business, we may encounter unforeseen expenses, difficulties, complications, delays and other known and unknown factors. We will need to transition from a company with a licensing and research focus to a company that is also capable of supporting clinical development and commercial activities. We may not be successful in such a transition.

Our ability to utilize our net operating loss carryforwards may be subject to limitations.

We have incurred substantial losses during our history and do not expect to become profitable in the near future and we may never achieve profitability. As of December 31, 2018, we had federal and state net operating loss carryforwards, or NOLs, of approximately $24.4 million and $24.0 million, respectively, which expire on various dates beginning in 2034 for those net operating loss carryforwards generated prior to 2018. Net operating losses generated in 2018 and beyond have no expiration. To the extent that we continue to generate taxable losses, unused losses will carry forward to offset future taxable income, if any, until such unused losses expire. Under Sections 382 and 383 of the Internal Revenue Code of 1986, as amended, or the Code, if a corporation undergoes an “ownership change,” generally defined as a greater than 50% change (by value) in its equity ownership over a three-year period, the corporation’s ability to use its pre-change NOLs and other pre-change tax attributes (such as research tax credits) to offset its post-change income may be limited. We may have experienced one or more ownership changes in prior years, and we may experience ownership changes in the future as a result of subsequent shifts in our stock ownership. As a result, if we earn net taxable income, our ability to use our pre-change NOLs to offset U.S. federal taxable income may be subject to limitations, which could potentially result in increased future tax liability to us. In addition, at the state level, there may be periods during which the use of NOLs is suspended or otherwise limited, which could accelerate or permanently increase state taxes owed.

U.S. federal income tax reform and changes in other tax laws could adversely affect us.

In December 2017, U.S. federal tax legislation, commonly referred to as the Tax Cuts and Jobs Act, or the TCJA, was signed into law, significantly reforming the Code. The TCJA, among other things, includes changes to U.S. federal tax rates, imposes significant additional limitations on the deductibility of business interest, allows for the expensing of capital expenditures, puts into effect the migration from a "worldwide" system of taxation to a partial "territorial" system, and modifies or repeals many business deductions and credits.

We continue to examine the impact the TCJA may have on our business. The TCJA is a far-reaching and complex revision to the U.S. federal income tax laws with disparate and, in some cases, countervailing impacts on different categories of taxpayers and industries, and will require subsequent rulemaking and interpretation in a number of areas. The long-term impact of the TCJA on the overall economy, the industries in which we operate and our and our partners' businesses cannot be reliably predicted at this early stage of the new law's implementation. There can be no assurance that the TCJA will not negatively impact our operating results, financial condition, and future business operations. The estimated impact of the TCJA is based on our management's current knowledge and assumptions, following consultation with our tax advisors. Because of our valuation allowance in the U.S., ongoing tax effects of the Act are not expected to materially change our effective tax rate in future periods.

In addition, new legislation or regulation which could affect our tax burden could be enacted by any governmental authority. We cannot predict the timing or extent of such tax-related developments which could have a negative impact on our financial results. Additionally, we use our best judgment in attempting to quantify and reserve for these tax obligations. However, a challenge by a taxing authority, our ability to utilize tax benefits such as carryforwards or tax credits, or a deviation from other tax-related assumptions could have a material adverse effect on our business, results of operations, or financial condition.

Risks related to our intellectual property

Our success depends in part on our ability to obtain, maintain and protect our intellectual property. It is difficult and costly to protect our proprietary rights and technology, and we may not be able to ensure their protection.

Our commercial success will depend in large part on obtaining and maintaining patent, trademark, trade secret and other intellectual property protection of our proprietary technologies and product candidates, which include TANGO, STK-001 and the additional gene targets identified by TANGO, their respective components, formulations, combination therapies, methods used to manufacture them and methods of treatment, as well as successfully defending our patents and other intellectual property rights against third-party challenges. Our ability to stop unauthorized third parties from making, using, selling, offering to sell, importing or otherwise commercializing our product candidates is dependent upon the extent to which we have rights under valid and enforceable patents or trade secrets that cover these activities. If we are unable to secure and maintain patent protection for any product or technology we develop, or if the scope of the patent protection secured is not sufficiently broad, our competitors could develop and commercialize products and technology similar or identical to ours, and our ability to commercialize any product candidates we may develop may be adversely affected.

The patenting process is expensive and time-consuming, and we may not be able to file and prosecute all necessary or desirable patent applications at a reasonable cost or in a timely manner. In addition, we may not pursue or obtain patent protection in all relevant markets. It is also possible that we will fail to identify patentable aspects of our research and development output before it is too late to obtain patent protection. Moreover, in some circumstances, we may not have the right to control the preparation, filing and prosecution of patent applications, or to maintain the patents, covering technology that we license from or license to third

parties and are reliant on our licensors or licensees to do so. Our pending and future patent applications may not result in issued patents. Even if patent applications we license or own currently or in the future issue as patents, they may not issue in a form that will provide us with any meaningful protection, prevent competitors or other third parties from competing with us, or otherwise provide us with any competitive advantage. Any patents that we hold or in-license may be challenged, narrowed, circumvented, or invalidated by third parties. Consequently, we do not know whether any of our platform advances and product candidates will be protectable or remain protected by valid and enforceable patents. In addition, our existing patents and any future patents we obtain may not be sufficiently broad to prevent others from using our technology or from developing competing products and technologies.

We depend on intellectual property licensed from third parties, and our licensors may not always act in our best interest. If we fail to comply with our obligations under our intellectual property licenses, if the licenses are terminated, or if disputes regarding these licenses arise, we could lose significant rights that are important to our business.

We are dependent on patents, know-how and proprietary technology licensed from others. Our licenses to such patents, know-how and proprietary technology may not provide exclusive rights in all relevant fields of use and in all territories in which we may wish to develop or commercialize our products in the future. The agreements under which we license patents, know-how and proprietary technology from others are complex, and certain provisions in such agreements may be susceptible to multiple interpretations.

For example, we are a party to license agreements with Cold Spring Harbor Laboratory and the University of Southampton, pursuant to which we in-license key patent and patent applications for our TANGO platform, STK-001 and future product candidates. For more information regarding these agreements, please see “Business—License agreements.” These agreements impose various diligence, milestone payment, royalty, insurance and other obligations on us. If we fail to comply with these obligations, our licensors may have the right to terminate our license, in which event we would not be able to develop or market our TANGO platform or STK-001 or any other technology or product candidates covered by the intellectual property licensed under these agreements. In addition, we may need to obtain additional licenses from our existing licensors and others to advance our research or allow commercialization of product candidates we may develop. It is possible that we may be unable to obtain any additional licenses at a reasonable cost or on reasonable terms, if at all. In either event, we may be required to expend significant time and resources to redesign our technology, product candidates, or the methods for manufacturing them or to develop or license replacement technology, all of which may not be feasible on a technical or commercial basis. If we are unable to do so, we may be unable to develop or commercialize the affected technology or product candidates.

If we or our licensors fail to adequately protect our licensed intellectual property, our ability to commercialize product candidates could suffer. We do not have complete control over the maintenance, prosecution and litigation of our in-licensed patents and patent applications and may have limited control over future intellectual property that may be in-licensed. For example, we cannot be certain that activities such as the maintenance and prosecution by our licensors have been or will be conducted in compliance with applicable laws and regulations or will result in valid and enforceable patents and other intellectual property rights. It is possible that our licensors’ infringement proceedings or defense activities may be less vigorous than had we conducted them ourselves, or may not be conducted in accordance with our best interests.

Furthermore, inventions contained within some of our in-licensed patents and patent applications were made using U.S. government funding or other non-governmental funding. We rely on our licensors to ensure compliance with applicable obligations arising from such funding, such as timely reporting, an obligation associated with in-licensed patents and patent applications. The failure of our licensors to meet their obligations may lead to a loss of rights or the unenforceability of relevant patents. For example, the

government could have certain rights in such in-licensed patents, including a non-exclusive license authorizing the government to use the invention or to have others use the invention on its behalf for non-commercial purposes. If the U.S. government then decides to exercise these rights, it is not required to engage us as its contractor in connection with doing so. These rights may also permit the government to exercise march-in rights to use or allow third parties to use the technology covered by such in-licensed patents. The government may also exercise its march-in rights if it determines that action is necessary because we or our licensors failed to achieve practical application of the government-funded technology, because action is necessary to alleviate health or safety needs, to meet requirements of federal regulations, or to give preference to U.S. industry. In addition, our rights in such in-licensed government-funded inventions may be subject to certain requirements to manufacture products embodying such inventions in the United States. Any of the foregoing could harm our business, financial condition, results of operations, and prospects significantly.

In addition, the resolution of any contract interpretation disagreement that may arise could narrow what we believe to be the scope of our rights to the relevant patents, know-how and proprietary technology, or increase what we believe to be our financial or other obligations under the relevant agreement. Disputes that may arise between us and our licensors regarding intellectual property subject to a license agreement could include disputes regarding:

- the scope of rights granted under the license agreement and other interpretation-related issues;
- whether and the extent to which our technology and processes infringe on intellectual property of the licensor that is not subject to the licensing agreement;
- our right to sublicense patent and other rights to third parties under collaborative development relationships;
- our diligence obligations with respect to the use of the licensed technology in relation to our development and commercialization of our product candidates and what activities satisfy those diligence obligations; and
- the ownership of inventions and know-how resulting from the joint creation or use of intellectual property by our licensors and us.

If disputes over intellectual property that we have licensed prevent or impair our ability to maintain our current licensing arrangements on acceptable terms, we may be unable to successfully develop and commercialize the affected technology or product candidates. As a result, any termination of or disputes over our intellectual property licenses could result in the loss of our ability to develop and commercialize our TANGO platform, or STK-001, or we could lose other significant rights, any of which could have a material adverse effect on our business, financial condition, results of operations, and prospects.

For example, our agreements with certain of our third-party research partners provide that improvements developed in the course of our relationship may be owned solely by either us or our third party research partner, or jointly between us and the third party. If we determine that rights to such improvements owned solely by a research partner or other third party with whom we collaborate are necessary to commercialize our drug candidates or maintain our competitive advantage, we may need to obtain a license from such third party in order to use the improvements and continue developing, manufacturing or marketing our drug candidates. We may not be able to obtain such a license on an exclusive basis, on commercially reasonable terms, or at all, which could prevent us from commercializing our drug candidates or allow our competitors or others the chance to access technology that is important to our business. We also may need the cooperation of any co-owners of our intellectual property in order to enforce such intellectual property against third parties, and such cooperation may not be provided to us.

Our owned and in-licensed patents and patent applications may not provide sufficient protection of our TANGO platform and our STK-001 product candidate and our future product candidates or result in any competitive advantage.

We have in-licensed an issued U.S. patent and patent applications that cover the mechanism of action of STK-001. As of the date of this prospectus, we have applied for patent applications intended to specifically cover STK-001 and its use, but do not currently own or in-license any issued U.S. patents that specifically cover STK-001 or its use. We cannot be certain that any of these patent applications will issue as patents, and if they do, that such patents will cover or adequately protect STK-001 or that such patents will not be challenged, narrowed, circumvented, invalidated or held unenforceable.

In addition to claims directed toward the technology underlying our TANGO platform, our owned and in-licensed patents and patent applications contain claims directed to compositions of matter on the active pharmaceutical ingredients, or APIs, in our product candidates, as well as methods-of-use directed to the use of an API for a specified treatment. Composition-of-matter patents on the active pharmaceutical ingredient in prescription drug products provide protection without regard to any particular method of use of the API used. Method-of-use patents do not prevent a competitor or other third party from developing or marketing an identical product for an indication that is outside the scope of the patented method. Moreover, with respect to method-of-use patents, even if competitors or other third parties do not actively promote their product for our targeted indications or uses for which we may obtain patents, providers may recommend that patients use these products off-label, or patients may do so themselves. Although off-label use may infringe or contribute to the infringement of method-of-use patents, the practice is common and this type of infringement is difficult to prevent or prosecute.

The strength of patents in the biotechnology and pharmaceutical field involves complex legal and scientific questions and can be uncertain. The patent applications that we own or in-license may fail to result in issued patents with claims that cover our product candidates or uses thereof in the United States or in other foreign countries. For example, while our patent applications are pending, we may be subject to a third party preissuance submission of prior art to the United States Patent and Trademark Office, or USPTO, or become involved in interference or derivation proceedings, or equivalent proceedings in foreign jurisdictions. Even if patents do successfully issue, third parties may challenge their inventorship, validity, enforceability or scope, including through opposition, revocation, reexamination, post-grant and *inter partes* review proceedings. An adverse determination in any such submission, proceeding or litigation may result in loss of patent rights, loss of exclusivity, or in patent claims being narrowed, invalidated, or held unenforceable, which could limit our ability to stop others from using or commercializing similar or identical technology and products, or limit the duration of the patent protection of our technology and product candidates. Furthermore, even if they are unchallenged, our patents and patent applications may not adequately protect our intellectual property or prevent others from designing around our claims. Moreover, some of our owned and in-licensed patents and patent applications may be co-owned with third parties. If we are unable to obtain an exclusive license to any such third-party co-owners' interest in such patents or patent applications, such co-owners may be able to license their rights to other third parties, including our competitors, and our competitors could market competing products and technology. In addition, we may need the cooperation of any such co-owners of our patents in order to enforce such patents against third parties, and such cooperation may not be provided to us. If the breadth or strength of protection provided by the patent applications we hold with respect to our product candidates is threatened, it could dissuade companies from collaborating with us to develop, and threaten our ability to commercialize, our product candidates. Further, if we encounter delays in development, testing, and regulatory review of new product candidates, the period of time during which we could market our product candidates under patent protection would be reduced.

Since patent applications in the United States and other countries are confidential for a period of time after filing, at any moment in time, we cannot be certain that we were in the past or will be in the future the first to file any patent application related to our product candidates. In addition, some patent applications in the United States may be maintained in secrecy until the patents are issued. As a result, there may be prior art of which we are not aware that may affect the validity or enforceability of a patent claim, and we may be subject to priority disputes. We may be required to disclaim part or all of the term of certain patents or all of the term of certain patent applications. There may be prior art of which we are not aware that may affect the validity or enforceability of a patent claim. There also may be prior art of which we are aware, but which we do not believe affects the validity or enforceability of a claim, which may, nonetheless, ultimately be found to affect the validity or enforceability of a claim. No assurance can be given that, if challenged, our patents would be declared by a court, patent office or other governmental authority to be valid or enforceable or that even if found valid and enforceable, a competitor's technology or product would be found by a court to infringe our patents. We may analyze patents or patent applications of our competitors that we believe are relevant to our activities, and consider that we are free to operate in relation to our product candidates, but our competitors may achieve issued claims, including in patents we consider to be unrelated, that block our efforts or potentially result in our product candidates or our activities infringing such claims. It is possible that our competitors may have filed, and may in the future file, patent applications covering our products or technology similar to ours. Those patent applications may have priority over our owned and in-licensed patent applications or patents, which could require us to obtain rights to issued patents covering such technologies. The possibility also exists that others will develop products that have the same effect as our product candidates on an independent basis that do not infringe our patents or other intellectual property rights, or will design around the claims of patents that we have had issued that cover our product candidates.

Likewise, our currently owned and in-licensed patents and patent applications, if issued as patents, directed to our proprietary technologies and our product candidates are expected to expire from 2035 through 2040, without taking into account any possible patent term adjustments or extensions. Our earliest in-licensed patents may expire before, or soon after, our first product achieves marketing approval in the United States or foreign jurisdictions. Additionally, we cannot be assured that the USPTO or relevant foreign patent offices will grant any of the pending patent applications we own or in-license currently or in the future. Upon the expiration of our current patents, we may lose the right to exclude others from practicing these inventions. The expiration of these patents could also have a similar material adverse effect on our business, financial condition, results of operations and prospects.

The degree of future protection for our proprietary rights is uncertain because legal means afford only limited protection and may not adequately protect our rights or permit us to gain or keep our competitive advantage. For example:

- others may be able to make or use compounds that are similar to the active compositions of our product candidates but that are not covered by the claims of our patents;
- the active pharmaceutical ingredients in our current product candidates will eventually become commercially available in generic drug products, and no patent protection may be available with regard to formulation or method of use;
- we or our licensors, as the case may be, may fail to meet our obligations to the U.S. government regarding any in-licensed patents and patent applications funded by U.S. government grants, leading to the loss or unenforceability of patent rights;
- we or our licensors, as the case may be, might not have been the first to file patent applications for certain inventions;

- others may independently develop similar or alternative technologies or duplicate any of our technologies;
- it is possible that our pending patent applications will not result in issued patents;
- it is possible that there are prior public disclosures that could invalidate our owned or in-licensed patents, as the case may be, or parts of our owned or in-licensed patents;
- it is possible that others may circumvent our owned or in-licensed patents;
- it is possible that there are unpublished applications or patent applications maintained in secrecy that may later issue with claims covering our product candidates or technology similar to ours;
- the laws of foreign countries may not protect our or our licensors', as the case may be, proprietary rights to the same extent as the laws of the United States;
- the claims of our owned or in-licensed issued patents or patent applications, if and when issued, may not cover our product candidates;
- our owned or in-licensed issued patents may not provide us with any competitive advantages, may be narrowed in scope, or be held invalid or unenforceable as a result of legal challenges by third parties;
- the inventors of our owned or in-licensed patents or patent applications may become involved with competitors, develop products or processes that design around our patents, or become hostile to us or the patents or patent applications on which they are named as inventors;
- it is possible that our owned or in-licensed patents or patent applications omit individual(s) that should be listed as inventor(s) or include individual(s) that should not be listed as inventor(s), which may cause these patents or patents issuing from these patent applications to be held invalid or unenforceable;
- we have engaged in scientific collaborations in the past and will continue to do so in the future and our collaborators may develop adjacent or competing products that are outside the scope of our patents;
- we may not develop additional proprietary technologies for which we can obtain patent protection;
- it is possible that product candidates or diagnostic tests we develop may be covered by third parties' patents or other exclusive rights; or
- the patents of others may have an adverse effect on our business.

Any of the foregoing could have a material adverse effect on our business, financial conditions, results of operations and prospects.

Our strategy of obtaining rights to key technologies through in-licenses may not be successful.

We seek to expand our product candidate pipeline in part by in-licensing the rights to key technologies, including those related to specific gene targets which may be upregulated by TANGO. The future growth of our business will depend in part on our ability to in-license or otherwise acquire the rights to additional product candidates and technologies. Although we have succeeded in licensing technologies from Cold Spring Harbor Laboratory and the University of Southampton in the past, we cannot assure you that we will be able to in-license or acquire the rights to any product candidates or technologies from third parties on acceptable terms or at all.

For example, our agreements with certain of our third party research partners provide that improvements developed in the course of our relationship may be owned solely by either us or our third party research partner, or jointly between us and the third party. If we determine that exclusive rights to such improvements

owned solely by a research partner or other third party with whom we collaborate are necessary to commercialize our drug candidates or maintain our competitive advantage, we may need to obtain an exclusive license from such third party in order to use the improvements and continue developing, manufacturing or marketing our drug candidates. We may not be able to obtain such a license on an exclusive basis, on commercially reasonable terms, or at all, which could prevent us from commercializing our drug candidates or allow our competitors or others the opportunity to access technology that is important to our business. We also may need the cooperation of any co-owners of our intellectual property in order to enforce such intellectual property against third parties, and such cooperation may not be provided to us.

In addition, the in-licensing and acquisition of these technologies is a highly competitive area, and a number of more established companies are also pursuing strategies to license or acquire product candidates or technologies that we may consider attractive. These established companies may have a competitive advantage over us due to their size, cash resources and greater clinical development and commercialization capabilities. In addition, companies that perceive us to be a competitor may be unwilling to license rights to us. Furthermore, we may be unable to identify suitable product candidates or technologies within our area of focus. If we are unable to successfully obtain rights to suitable product candidates or technologies, our business and prospects could be materially and adversely affected.

If we are unable to protect the confidentiality of our trade secrets, our business and competitive position would be harmed.

In addition to patent protection, we rely upon know-how and trade secret protection, as well as non-disclosure agreements and invention assignment agreements with our employees, consultants and third-parties, to protect our confidential and proprietary information, especially where we do not believe patent protection is appropriate or obtainable.

It is our policy to require our employees, consultants, outside scientific collaborators, sponsored researchers and other advisors to execute confidentiality agreements upon the commencement of employment or consulting relationships with us. These agreements provide that all confidential information concerning our business or financial affairs developed or made known to the individual or entity during the course of the party's relationship with us is to be kept confidential and not disclosed to third parties, except in certain specified circumstances. In the case of employees, the agreements provide that all inventions conceived by the individual, and that are related to our current or planned business or research and development or made during normal working hours, on our premises or using our equipment or proprietary information, are our exclusive property. In the case of consultants and other third parties, the agreements provide that all inventions conceived in connection with the services provided are our exclusive property. However, we cannot guarantee that we have entered into such agreements with each party that may have or have had access to our trade secrets or proprietary technology and processes. We have also adopted policies and conduct training that provides guidance on our expectations, and our advice for best practices, in protecting our trade secrets. Despite these efforts, any of these parties may breach the agreements and disclose our proprietary information, including our trade secrets, and we may not be able to obtain adequate remedies for such breaches.

In addition to contractual measures, we try to protect the confidential nature of our proprietary information through other appropriate precautions, such as physical and technological security measures. However, trade secrets and know-how can be difficult to protect. These measures may not, for example, in the case of misappropriation of a trade secret by an employee or third party with authorized access, provide adequate protection for our proprietary information. Our security measures may not prevent an employee or consultant from misappropriating our trade secrets and providing them to a competitor, and any recourse we might take against this type of misconduct may not provide an adequate remedy to protect our interests fully. Enforcing a

claim that a party illegally disclosed or misappropriated a trade secret can be difficult, expensive, and time-consuming, and the outcome is unpredictable. In addition, trade secrets may be independently developed by others in a manner that could prevent us from receiving legal recourse. If any of our confidential or proprietary information, such as our trade secrets, were to be disclosed or misappropriated, or if any of that information was independently developed by a competitor, our competitive position could be harmed.

In addition, courts outside the United States are sometimes less willing to protect trade secrets. If we choose to go to court to stop a third party from using any of our trade secrets, we may incur substantial costs. Even if we are successful, these types of lawsuits may consume our time and other resources. Although we take steps to protect our proprietary information and trade secrets, third parties may independently develop substantially equivalent proprietary information and techniques or otherwise gain access to our trade secrets or disclose our technology. As a result, we may not be able to meaningfully protect our trade secrets. Any of the foregoing could have a material adverse effect on our business, financial condition, results of operations and prospects.

Third-party claims of intellectual property infringement may prevent, delay or otherwise interfere with our product discovery and development efforts.

Our commercial success depends in part on our ability to develop, manufacture, market and sell our product candidates and use our proprietary technologies without infringing, misappropriating or otherwise violating the intellectual property or proprietary rights of third parties. There is a substantial amount of litigation involving patents and other intellectual property rights in the biotechnology and pharmaceutical industries, as well as administrative proceedings for challenging patents, including interference, derivation, *inter partes* review, post grant review, and reexamination proceedings before the USPTO or oppositions and other comparable proceedings in foreign jurisdictions. We may be exposed to, or threatened with, future litigation by third parties having patent or other intellectual property rights alleging that our product candidates and/or proprietary technologies infringe, misappropriate or otherwise violate their intellectual property rights. Numerous U.S. and foreign issued patents and pending patent applications that are owned by third parties exist in the fields in which we are developing our product candidates. As the biotechnology and pharmaceutical industries expand and more patents are issued, the risk increases that our product candidates may give rise to claims of infringement of the patent rights of others. Moreover, it is not always clear to industry participants, including us, which patents cover various types of drugs, products or their methods of use or manufacture. Thus, because of the large number of patents issued and patent applications filed in our field, third parties may allege they have patent rights encompassing our product candidates, technologies or methods.

If a third party claims that we infringe, misappropriate or otherwise violate its intellectual property rights, we may face a number of issues, including, but not limited to:

- infringement and other intellectual property claims that, regardless of merit, may be expensive and time-consuming to litigate and may divert our management's attention from our core business;
- substantial damages for infringement, which we may have to pay if a court decides that the product candidate or technology at issue infringes on or violates the third party's rights, and, if the court finds that the infringement was willful, we could be ordered to pay treble damages plus the patent owner's attorneys' fees;
- a court prohibiting us from developing, manufacturing, marketing or selling our product candidates, or from using our proprietary technologies, unless the third party licenses its product rights to us, which it is not required to do, on commercially reasonable terms or at all;
- if a license is available from a third party, we may have to pay substantial royalties, upfront fees and other amounts, and/or grant cross-licenses to intellectual property rights for our product candidates;

- the requirement that we redesign our product candidates or processes so they do not infringe, which may not be possible or may require substantial monetary expenditures and time; and
- there could be public announcements of the results of hearings, motions, or other interim proceedings or developments, and if securities analysts or investors perceive these results to be negative, it could have a substantial adverse effect on the price of our common stock.

Some of our competitors may be able to sustain the costs of complex patent litigation more effectively than we can because they have substantially greater resources. In addition, any uncertainties resulting from the initiation and continuation of any litigation could have a material adverse effect on our ability to raise the funds necessary to continue our operations or could otherwise have a material adverse effect on our business, financial condition, results of operations and prospects.

Third parties may assert that we are employing their proprietary technology without authorization, including by enforcing its patents against us by filing a patent infringement lawsuit against us. In this regard, patents issued in the United States by law enjoy a presumption of validity that can be rebutted only with evidence that is “clear and convincing,” a heightened standard of proof.

There may be third-party patents of which we are currently unaware with claims to materials, formulations, methods of manufacture or methods for treatment related to the use or manufacture of our product candidates. Because patent applications can take many years to issue, there may be currently pending patent applications that may later result in issued patents that our product candidates may infringe. In addition, third parties may obtain patents in the future and claim that use of our technologies infringes upon these patents.

If any third-party patents were held by a court of competent jurisdiction to cover the manufacturing process of our product candidates, or materials used in or formed during the manufacturing process, or any final product itself, the holders of those patents may be able to block our ability to commercialize our product candidate unless we obtain a license under the applicable patents, or until those patents were to expire or those patents are finally determined to be invalid or unenforceable. Similarly, if any third-party patent were held by a court of competent jurisdiction to cover aspects of our formulations, processes for manufacture or methods of use, including combination therapy or patient selection methods, the holders of that patent may be able to block our ability to develop and commercialize the product candidate unless we obtain a license or until such patent expires or is finally determined to be invalid or unenforceable. In either case, a license may not be available on commercially reasonable terms, or at all, particularly if such patent is owned or controlled by one of our primary competitors. If we are unable to obtain a necessary license to a third-party patent on commercially reasonable terms, or at all, our ability to commercialize our product candidates may be impaired or delayed, which could significantly harm our business. Even if we obtain a license, it may be non-exclusive, thereby giving our competitors access to the same technologies licensed to us. In addition, if the breadth or strength of protection provided by our patents and patent applications is threatened, it could dissuade companies from collaborating with us to license, develop or commercialize current or future product candidates.

Parties making claims against us may seek and obtain injunctive or other equitable relief, which could effectively block our ability to further develop and commercialize our product candidates. Defense of these claims, regardless of their merit, would involve substantial litigation expense and would be a substantial diversion of employee time and resources from our business. In the event of a successful claim of infringement against us, we may have to pay substantial damages, including treble damages and attorneys’ fees for willful infringement, obtain one or more licenses from third parties, pay royalties or redesign our infringing products, which may be impossible or require substantial time and monetary expenditure. We cannot predict whether any license of this nature would be available at all or whether it would be available on commercially reasonable terms. Furthermore, even in the absence of litigation, we may need to obtain licenses from third parties to

advance our research or allow commercialization of our product candidates and we may fail to obtain any of these licenses at a reasonable cost or on reasonable terms, if at all. In that event, we would be unable to further develop and commercialize our product candidates, which could significantly harm our business.

We may be involved in lawsuits to protect or enforce our patents or the patents of our licensors, which could be expensive, time-consuming and unsuccessful and could result in a finding that such patents are unenforceable or invalid.

Competitors may infringe our patents or the patents of our licensors. To counter infringement or unauthorized use, we may be required to file infringement claims, which can be expensive and time-consuming. In addition, in an infringement proceeding, a court may decide that one or more of our patents is not valid or is unenforceable, or may refuse to stop the other party from using the technology at issue on the grounds that our patents do not cover the technology in question.

In patent litigation in the United States, defendant counterclaims alleging invalidity and/or unenforceability are commonplace, and there are numerous grounds upon which a third party can assert invalidity or unenforceability of a patent. Third parties may also raise similar claims before administrative bodies in the United States or abroad, even outside the context of litigation. These types of mechanisms include re-examination, post-grant review, *inter partes* review, interference proceedings, derivation proceedings, and equivalent proceedings in foreign jurisdictions (e.g., opposition proceedings). These types of proceedings could result in revocation or amendment to our patents such that they no longer cover our product candidates. The outcome for any particular patent following legal assertions of invalidity and unenforceability is unpredictable. With respect to the validity question, for example, we cannot be certain that there is no invalidating prior art, of which we, our patent counsel and the patent examiner were unaware during prosecution. If a defendant were to prevail on a legal assertion of invalidity and/or unenforceability, or if we are otherwise unable to adequately protect our rights, we would lose at least part, and perhaps all, of the patent protection on our product candidates. Defense of these types of claims, regardless of their merit, would involve substantial litigation expense and would be a substantial diversion of employee resources from our business.

Conversely, we may choose to challenge the patentability of claims in a third party's U.S. patent by requesting that the USPTO review the patent claims in re-examination, post-grant review, *inter partes* review, interference proceedings, derivation proceedings, and equivalent proceedings in foreign jurisdictions (e.g., opposition proceedings), or we may choose to challenge a third party's patent in patent opposition proceedings in the European Patent Office, or EPO, or another foreign patent office. Even if successful, the costs of these opposition proceedings could be substantial, and may consume our time or other resources. If we fail to obtain a favorable result at the USPTO, EPO or other patent office then we may be exposed to litigation by a third party alleging that the patent may be infringed by our product candidates or proprietary technologies.

Furthermore, because of the substantial amount of discovery required in connection with intellectual property litigation, there is a risk that some of our confidential information could be compromised by disclosure during this type of litigation. In addition, there could be public announcements of the results of hearings, motions or other interim proceedings or developments. If securities analysts or investors perceive these results to be negative, that perception could have a substantial adverse effect on the price of our common stock. Any of the foregoing could have a material adverse effect on our business financial condition, results of operations and prospects.

We have limited foreign intellectual property rights and may not be able to protect our intellectual property rights throughout the world.

We have limited intellectual property rights outside the United States. Filing, prosecuting and defending patents on product candidates in all countries throughout the world would be prohibitively expensive, and our

intellectual property rights in some countries outside the United States can be less extensive than those in the United States. In addition, the laws of some foreign countries do not protect intellectual property rights to the same extent as federal and state laws in the United States. Consequently, we may not be able to prevent third parties from practicing our inventions in all countries outside the United States, or from selling or importing products made using our inventions in and into the United States or other jurisdictions. Competitors may use our technologies in jurisdictions where we have not obtained patent protection to develop their own products and, further, may export otherwise infringing products to territories where we have patent protection but where enforcement is not as strong as that in the United States. These products may compete with our product candidates in jurisdictions where we do not have any issued patents and our patent claims or other intellectual property rights may not be effective or sufficient to prevent them from competing.

Many companies have encountered significant problems in protecting and defending intellectual property rights in foreign jurisdictions. The legal systems of certain countries, particularly certain developing countries, do not favor the enforcement of patents, trade secrets and other intellectual property protection, particularly those relating to biopharmaceutical products, which could make it difficult for us to stop the infringement of our patents or marketing of competing products against third parties in violation of our proprietary rights generally. The initiation of proceedings by third parties to challenge the scope or validity of our patent rights in foreign jurisdictions could result in substantial cost and divert our efforts and attention from other aspects of our business. Proceedings to enforce our patent rights in foreign jurisdictions could result in substantial costs and divert our efforts and attention from other aspects of our business, could put our patents at risk of being invalidated or interpreted narrowly and our patent applications at risk of not issuing and could provoke third parties to assert claims against us. We may not prevail in any lawsuits that we initiate and the damages or other remedies awarded, if any, may not be commercially meaningful. Accordingly, our efforts to enforce our intellectual property rights around the world may be inadequate to obtain a significant commercial advantage from the intellectual property that we develop or license.

Our use of open source software could impose limitations on our ability to commercialize our product candidates.

Our use of open source software could impose limitations on our ability to commercialize our product candidates. Our technology utilizes open source software that contains modules licensed for use from third-party authors under open source licenses. In particular, some of the software that powers TANGO may be provided under license arrangements that allow use of the software for research or other non-commercial purposes. As a result, in the future, as we seek to use our platform in connection with commercially available products, we may be required to license that software under different license terms, which may not be possible on commercially reasonable terms, if at all. If we are unable to license software components on terms that permit its use for commercial purposes, we may be required to replace those software components, which could result in delays, additional cost and additional regulatory approvals.

Use and distribution of open source software may entail greater risks than use of third-party commercial software, as open source licensors generally do not provide warranties or other contractual protections regarding infringement claims or the quality of the software code. Some open source licenses contain requirements that we make available source code for modifications or derivative works we create based upon the type of open source software we use. If we combine our proprietary software with open source software in a certain manner, we could, under certain of the open source licenses, be required to release the source code of our proprietary software to the public. This could allow our competitors to create similar products with lower development effort and time, and ultimately could result in a loss of product sales for us. Although we monitor our use of open source software, the terms of many open source licenses have not been interpreted by U.S. courts, and there is a risk that those licenses could be construed in a manner that could impose unanticipated conditions or restrictions on our ability to commercialize our product candidates. We could be required to seek

licenses from third parties in order to continue offering our product candidates, to re-engineer our product candidates or to discontinue the sale of our product candidates in the event re-engineering cannot be accomplished on a timely basis, any of which could materially and adversely affect our business, financial condition, results of operations and prospects.

Third parties may assert that our employees or consultants have wrongfully used or disclosed confidential information or misappropriated trade secrets.

As is common in the biotechnology and pharmaceutical industries, we employ individuals who were previously employed at universities or other biopharmaceutical or pharmaceutical companies, including our competitors or potential competitors. Although no misappropriation or improper disclosure claims against us are currently pending, and although we try to ensure that our employees and consultants do not use the proprietary information or know-how of others in their work for us, we may be subject to claims that we or our employees, consultants or independent contractors have inadvertently or otherwise used or disclosed intellectual property, including trade secrets or other proprietary information, of a former employer or other third parties. We may then have to pursue litigation to defend against these claims. If we fail in defending any claims of this nature in addition to paying monetary damages, we may lose valuable intellectual property rights or personnel. Even if we are successful in defending against these types of claims, litigation or other legal proceedings relating to intellectual property claims may cause us to incur significant expenses, and could distract our technical and management personnel from their normal responsibilities. In addition, there could be public announcements of the results of hearings, motions or other interim proceedings or developments, and, if securities analysts or investors perceive these results to be negative, that perception could have a substantial adverse effect on the price of our common stock. This type of litigation or proceeding could substantially increase our operating losses and reduce our resources available for development activities, and we may not have sufficient financial or other resources to adequately conduct this type of litigation or proceedings. For example, some of our competitors may be able to sustain the costs of this type of litigation or proceedings more effectively than we can because of their substantially greater financial resources. In any case, uncertainties resulting from the initiation and continuation of intellectual property litigation or other intellectual property related proceedings could adversely affect our ability to compete in the marketplace.

We may not be successful in obtaining or maintaining necessary rights to product components and processes for our development pipeline through acquisitions and in-licenses.

The growth of our business may depend in part on our ability to acquire, in-license or use third-party proprietary rights.

For example, our product candidates may require specific formulations to work effectively and efficiently, we may develop product candidates containing our compounds and pre-existing pharmaceutical compounds, or we may be required by the FDA or comparable foreign regulatory authorities to provide a companion diagnostic test or tests with our product candidates, any of which could require us to obtain rights to use intellectual property held by third parties. In addition, with respect to any patents we may co-own with third parties, we may require licenses to such co-owners interest to such patents. We may be unable to acquire or in-license any compositions, methods of use, processes or other third-party intellectual property rights from third parties that we identify as necessary or important to our business operations. In addition, we may fail to obtain any of these licenses at a reasonable cost or on reasonable terms, if at all. Were that to happen, we may need to cease use of the compositions or methods covered by those third-party intellectual property rights, and may need to seek to develop alternative approaches that do not infringe on those intellectual property rights, which may entail additional costs and development delays, even if we were able to develop such alternatives, which may not be feasible. Even if we are able to obtain a license, it may be non-exclusive, which means that our competitors may

also receive access to the same technologies licensed to us. In that event, we may be required to expend significant time and resources to develop or license replacement technology.

Additionally, we sometimes collaborate with academic institutions to accelerate our preclinical research or development under written agreements with these institutions. In certain cases, these institutions provide us with an option to negotiate a license to any of the institution's rights in technology resulting from the collaboration. Even if we hold such an option, we may be unable to negotiate a license from the institution within the specified timeframe or under terms that are acceptable to us. If we are unable to do so, the institution may offer the intellectual property rights to others, potentially blocking our ability to pursue our program.

The licensing and acquisition of third-party intellectual property rights is a competitive area, and companies that may be more established or have greater resources than we do may also be pursuing strategies to license or acquire third-party intellectual property rights that we may consider necessary or attractive in order to commercialize our product candidates. More established companies may have a competitive advantage over us due to their size, cash resources and greater clinical development and commercialization capabilities. In addition, companies that perceive us to be a competitor may be unwilling to assign or license rights to us. There can be no assurance that we will be able to successfully complete these types of negotiations and ultimately acquire the rights to the intellectual property surrounding the additional product candidates that we may seek to develop or market. If we are unable to successfully obtain rights to required third-party intellectual property or to maintain the existing intellectual property rights we have, we may have to abandon development of certain programs and our business financial condition, results of operations and prospects could suffer.

Obtaining and maintaining our patent protection depends on compliance with various procedural, document submission, fee payment and other requirements imposed by governmental patent agencies, and our patent protection could be reduced or eliminated for non-compliance with these requirements.

Periodic maintenance fees on any issued patent are due to be paid to the USPTO and foreign patent agencies in several stages over the lifetime of the patent. The USPTO and various foreign patent agencies also require compliance with a number of procedural, documentary, fee payment and other provisions during the patent application process and following the issuance of a patent. While an inadvertent lapse can in many cases be cured by payment of a late fee or by other means in accordance with the applicable rules, there are situations in which noncompliance can result in abandonment or lapse of the patent or patent application, resulting in partial or complete loss of patent rights in the relevant jurisdiction. Noncompliance events that could result in abandonment or lapse of a patent or patent application include, but are not limited to, failure to respond to official actions within prescribed time limits, non-payment of fees and failure to properly legalize and submit formal documents. Were a noncompliance event to occur, our competitors might be able to enter the market, which would have a material adverse effect on our business financial condition, results of operations and prospects.

Changes in patent law in the United States and in non-U.S. jurisdictions could diminish the value of patents in general, thereby impairing our ability to protect our product candidates.

As is the case with other biopharmaceutical companies, our success is heavily dependent on intellectual property, particularly patents. Obtaining and enforcing patents in the biopharmaceutical industry involve both technological and legal complexity, and is therefore costly, time-consuming and inherently uncertain.

Past or future patent reform legislation could increase the uncertainties and costs surrounding the prosecution of our patent applications and the enforcement or defense of our issued patents. For example, in March 2013, under the Leahy-Smith America Invents Act, or America Invents Act, the United States moved from a "first to invent" to a "first-to-file" patent system. Under a "first-to-file" system, assuming the other requirements for

patentability are met, the first inventor to file a patent application generally will be entitled to a patent on the invention regardless of whether another inventor had made the invention earlier. The America Invents Act includes a number of other significant changes to U.S. patent law, including provisions that affect the way patent applications are prosecuted, redefine prior art and establish a new post-grant review system. The effects of these changes are currently unclear as the USPTO continues to promulgate new regulations and procedures in connection with the America Invents Act and many of the substantive changes to patent law, including the “first-to-file” provisions, only became effective in March 2013. In addition, the courts have yet to address many of these provisions and the applicability of the act and new regulations on the specific patents discussed in this filing have not been determined and would need to be reviewed. However, the America Invents Act and its implementation could increase the uncertainties and costs surrounding the prosecution of our patent applications and the enforcement or defense of our issued patents.

Additionally, recent U.S. Supreme Court rulings have narrowed the scope of patent protection available in certain circumstances and weakened the rights of patent owners in certain situations. In addition to increasing uncertainty with regard to our ability to obtain patents in the future, this combination of events has created uncertainty with respect to the value of patents, once obtained. Depending on decisions by the U.S. Congress, the federal courts and the USPTO, the laws and regulations governing patents could change in unpredictable ways that would weaken our ability to obtain new patents or to enforce our existing patents and patents that we might obtain in the future. For example, in the case, *Assoc. for Molecular Pathology v. Myriad Genetics, Inc.,* the U.S. Supreme Court held that certain claims to DNA molecules are not patentable. While we do not believe that any of our owned or in-licensed patents will be found invalid based on this decision, we cannot predict how future decisions by the courts, the U.S. Congress or the USPTO may impact the value of our patents. Any similar adverse changes in the patent laws of other jurisdictions could also have a material adverse effect on our business, financial condition, results of operations and prospects.

Patent terms may be inadequate to protect our competitive position on our product candidates for an adequate amount of time.

Patents have a limited lifespan. In the United States, if all maintenance fees are timely paid, the natural expiration of a patent is generally 20 years from its earliest U.S. non-provisional filing date. Various extensions may be available, but the life of a patent, and the protection it affords, is limited. Even if patents covering our product candidates are obtained, once the patent life has expired, we may be open to competition from competitive products, including generics. Given the amount of time required for the development, testing and regulatory review of new product candidates, patents protecting our product candidates might expire before or shortly after we or our partners commercialize those candidates. As a result, our owned and licensed patent portfolio may not provide us with sufficient rights to exclude others from commercializing products similar or identical to ours.

If we do not obtain patent term extension and data exclusivity for any product candidates we may develop, our business may be materially harmed.

Depending upon the timing, duration and specifics of any FDA marketing approval of any product candidates we may develop, one or more of our U.S. patents may be eligible for limited patent term extension under the Drug Price Competition and Patent Term Restoration Act of 1984, or the Hatch-Waxman Amendments. The Hatch-Waxman Amendments permit a patent extension term of up to five years as compensation for patent term lost during the FDA regulatory review process. A patent term extension cannot extend the remaining term of a patent beyond a total of 14 years from the date of product approval, only one patent per product may be extended and only those claims covering the approved drug, a method for using it, or a method for manufacturing it may be extended. However, even if we were to seek a patent term extension, it may not be granted because of, for example, the failure to exercise due diligence during the testing phase or regulatory

review process, the failure to apply within applicable deadlines, the failure to apply prior to expiration of relevant patents, or any other failure to satisfy applicable requirements. Moreover, the applicable time period or the scope of patent protection afforded could be less than we request. If we are unable to obtain patent term extension or term of any such extension is less than we request, our competitors may obtain approval of competing products following our patent expiration, and our business, financial condition, results of operations, and prospects could be materially harmed.

We are subject to a variety of privacy and data security laws, and our failure to comply with them could harm our business.

We maintain a large quantity of sensitive information, including confidential business and patient health information in connection with our preclinical studies, and are subject to laws and regulations governing the privacy and security of such information. In the United States, there are numerous federal and state privacy and data security laws and regulations governing the collection, use, disclosure and protection of personal information, including federal and state health information privacy laws, federal and state security breach notification laws, and federal and state consumer protection laws. Each of these laws is subject to varying interpretations and constantly evolving. In May 2018, a new privacy regime, the General Data Protection Regulation, the GDPR, took effect in the European Economic Area, the EEA. The GDPR governs the collection, use, disclosure, transfer or other processing of personal data of European persons. Among other things, the GDPR imposes new requirements regarding the security of personal data and notification of data processing obligations to the competent national data processing authorities, changes the lawful bases on which personal data can be processed, expands the definition of personal data and requires changes to informed consent practices, as well as more detailed notices for clinical trial subjects and investigators. In addition, the GDPR increases the scrutiny of transfers of personal data from clinical trial sites located in the EEA to the United States and other jurisdictions that the European Commission does not recognize as having “adequate” data protection laws, and imposes substantial fines for breaches and violations (up to the greater of €20 million or 4% of our consolidated annual worldwide gross revenue). The GDPR also confers a private right of action on data subjects and consumer associations to lodge complaints with supervisory authorities, seek judicial remedies and obtain compensation for damages resulting from violations of the GDPR. Compliance with these and any other applicable privacy and data security laws and regulations is a rigorous and time-intensive process, and we may be required to put in place additional mechanisms ensuring compliance with the new data protection rules. If we fail to comply with any such laws or regulations, we may face significant fines and penalties that could adversely affect our business, financial condition and results of operations.

Risks related to employee matters, managing growth and other risks related to our business

We expect to expand our development and regulatory capabilities, and as a result, we may encounter difficulties in managing our growth, which could disrupt our operations.

We expect to experience significant growth in the number of our employees and the scope of our operations, particularly in the areas of product candidate development and growing our capability to conduct clinical trials. To manage our anticipated future growth, we must continue to implement and improve our managerial, operational and financial systems, expand our facilities and continue to recruit and train additional qualified personnel. Due to our limited financial resources and the limited experience of our management team in managing a company with such anticipated growth, we may not be able to effectively manage the expansion of our operations or recruit and train additional qualified personnel. The expansion of our operations may lead to significant costs and may divert our management and business development resources. Any inability to manage growth could delay the execution of our business plans or disrupt our operations.

We must attract and retain highly skilled employees to succeed.

To succeed, we must recruit, retain, manage and motivate qualified clinical, scientific, technical and management personnel, and we face significant competition for experienced personnel. If we do not succeed in attracting and retaining qualified personnel, particularly at the management level, it could adversely affect our ability to execute our business plan, harm our results of operations and increase our capabilities to successfully commercialize STK-001 and our future product candidates. In particular, we believe that our future success is highly dependent upon the contributions of our senior management, particularly our Chief Executive Officer, Edward M. Kaye, M.D., our Chief Operating Officer and Chief Business Officer, Huw M. Nash, Ph.D., our Chief Medical Officer, Barry S. Ticho, M.D., Ph.D., FACC, and our Co-Founder and Vice President, Head of Biology, Isabel Aznarez, as well as our senior scientists and other members of our senior management team. The loss of services of any of these individuals, who all have at-will employment arrangements with us, could delay or prevent the successful development of our product pipeline, completion of our planned clinical trials or the commercialization of our product candidates, if approved. The competition for qualified personnel in the biotechnology field is intense and as a result, we may be unable to continue to attract and retain qualified personnel necessary for the development of our business or to recruit suitable replacement personnel. In addition, certain members of our senior management team, including our Chief Financial Officer, who joined us in March 2019, have worked together for only a relatively short period of time and it may be difficult to evaluate their effectiveness, on an individual or collective basis, and ability to address future challenges to our business.

Many of the other biotechnology companies that we compete against for qualified personnel have greater financial and other resources, different risk profiles and a longer history in the industry than we do. They also may provide more diverse opportunities and better chances for career advancement. Some of these characteristics may be more appealing to high-quality candidates than what we have to offer. If we are unable to continue to attract and retain high-quality personnel, the rate and success at which we can discover and develop product candidates and our business will be limited.

Future acquisitions or strategic alliances could disrupt our business and harm our financial condition and results of operations.

We may acquire additional businesses or drugs, form strategic alliances or create joint ventures with third parties that we believe will complement or augment our existing business. If we acquire businesses with promising markets or technologies, we may not be able to realize the benefit of acquiring such businesses if we are unable to successfully integrate them with our existing operations and company culture. We may encounter numerous difficulties in developing, manufacturing and marketing any new drugs resulting from a strategic alliance or acquisition that delay or prevent us from realizing their expected benefits or enhancing our business. We cannot assure you that, following any such acquisition, we will achieve the expected synergies to justify the transaction. The risks we face in connection with acquisitions, include:

- diversion of management time and focus from operating our business to addressing acquisition integration challenges;
- coordination of research and development efforts;
- retention of key employees from the acquired company;
- changes in relationships with strategic partners as a result of product acquisitions or strategic positioning resulting from the acquisition;
- cultural challenges associated with integrating employees from the acquired company into our organization;
- the need to implement or improve controls, procedures, and policies at a business that prior to the acquisition may have lacked sufficiently effective controls, procedures and policies;

- liability for activities of the acquired company before the acquisition, including intellectual property infringement claims, violation of laws, commercial disputes, tax liabilities, and other known liabilities;
- unanticipated write-offs or charges; and
- litigation or other claims in connection with the acquired company, including claims from terminated employees, customers, former stockholders or other third parties.

Our failure to address these risks or other problems encountered in connection with our past or future acquisitions or strategic alliances could cause us to fail to realize the anticipated benefits of these transactions, cause us to incur unanticipated liabilities and harm the business generally. There is also a risk that future acquisitions will result in the incurrence of debt, contingent liabilities, amortization expenses or incremental operating expenses, any of which could harm our financial condition or results of operations.

If we fail to comply with environmental, health, and safety laws and regulations, we could become subject to fines or penalties or incur costs that could harm our business.

We will become subject to numerous environmental, health, and safety laws and regulations, including those governing laboratory procedures and the handling, use, storage, treatment, and disposal of hazardous materials and wastes. Our operations will involve the use of hazardous and flammable materials, including chemicals and biological materials. Our operations also may produce hazardous waste products. We generally anticipate contracting with third parties for the disposal of these materials and wastes. We will not be able to eliminate the risk of contamination or injury from these materials. In the event of contamination or injury resulting from any use by us of hazardous materials, we could be held liable for any resulting damages, and any liability could exceed our resources. We also could incur significant costs associated with civil or criminal fines and penalties for failure to comply with such laws and regulations.

Although we maintain workers' compensation insurance to cover us for costs and expenses we may incur due to injuries to our employees resulting from the use of hazardous materials, this insurance may not provide adequate coverage against potential liabilities.

In addition, we may incur substantial costs in order to comply with current or future environmental, health, and safety laws and regulations. These current or future laws and regulations may impair our research, development, or production efforts. Our failure to comply with these laws and regulations also may result in substantial fines, penalties or other sanctions.

Unfavorable global economic conditions could adversely affect our business, financial condition, stock price and results of operations.

Our results of operations could be adversely affected by general conditions in the global economy and in the global financial markets. For example, the global financial crisis caused extreme volatility and disruptions in the capital and credit markets. A severe or prolonged economic downturn, such as the 2008 global financial crisis, could result in a variety of risks to our business, including, weakened demand for our product candidates and our ability to raise additional capital when needed on acceptable terms, if at all. A weak or declining economy could also strain our suppliers, possibly resulting in supply disruption, or cause our customers to delay making payments for our services. If the current equity and credit markets deteriorate, it may make any necessary debt or equity financing more difficult, more costly, and more dilutive. Failure to secure any necessary financing in a timely manner and on favorable terms could have a material adverse effect on our growth strategy, financial performance and stock price and could require us to delay or abandon clinical development plans. In addition, there is a risk that one or more of our current service providers, manufacturers and other partners may not survive such difficult economic times, which could directly affect our ability to attain our operating goals on

schedule and on budget. Any of the foregoing could harm our business and we cannot anticipate all of the ways in which the current economic climate and financial market conditions could adversely impact our business. Furthermore, our stock price may decline due in part to the volatility of the stock market and any general economic downturn.

We or the third parties upon whom we depend may be adversely affected by natural disasters and our business continuity and disaster recovery plans may not adequately protect us from a serious disaster.

Natural disasters could severely disrupt our operations and have a material adverse effect on our business, results of operations, financial condition and prospects. If a natural disaster, fire, hurricane, power outage or other event occurred that prevented us from using all or a significant portion of our headquarters, that damaged critical infrastructure, such as our suppliers' manufacturing facilities, or that otherwise disrupted operations, it may be difficult or, in certain cases, impossible for us to continue our business for a substantial period of time.

The disaster recovery and business continuity plans we have in place may prove inadequate in the event of a serious disaster or similar event. We may incur substantial expenses as a result of the limited nature of our disaster recovery and business continuity plans, which could have a material adverse effect on our business.

Our internal computer and information systems, or those used by our CROs, CMOs or other contractors or consultants, may fail or suffer security breaches, which could result in a material disruption of our development programs.

Despite the implementation of appropriate security measures, our internal computer and information systems and those of our current and any future CROs, CMOs and other contractors or consultants may become vulnerable to damage from computer viruses, unauthorized access, natural disasters, terrorism, war and telecommunication and electrical failures. While we have not experienced any such material system failure, or accident, and are unaware of any security breach to date, if such an event were to occur and cause interruptions in our operations, it could result in a material disruption of our development programs and our business operations, whether due to a loss of our trade secrets or other proprietary information or other similar disruptions. For example, the loss of data from completed or future preclinical studies or clinical trials could result in significant delays in our regulatory approval efforts and significantly increase our costs to recover or reproduce the data. To the extent that any disruption or security breach were to result in a loss of, or damage to, our data or applications, or inappropriate disclosure of confidential or proprietary information, we could incur liability, our competitive position could be harmed and the further development and commercialization of our product candidates could be significantly delayed.

We may be unable to adequately protect our information systems from cyberattacks, which could result in the disclosure of confidential information, damage our reputation, and subject us to significant financial and legal exposure.

Cyberattacks are increasing in their frequency, sophistication and intensity, and have become increasingly difficult to detect. Cyberattacks could include wrongful conduct by hostile foreign governments, industrial espionage, wire fraud and other forms of cyber fraud, the deployment of harmful malware, denial-of-service, social engineering fraud or other means to threaten data confidentiality, integrity and availability. A successful cyberattack could cause serious negative consequences for us, including, without limitation, the disruption of operations, the misappropriation of confidential business information, including financial information, trade secrets, financial loss and the disclosure of corporate strategic plans. To date, we have not experienced a material compromise of our data or information systems. However, although we devote resources to protect our information systems, we realize that cyberattacks are a threat, and there can be no assurance that our efforts will prevent information security breaches that would result in business, legal, financial or reputational harm to us, or would have a material adverse effect on our results of operations and financial condition.

In addition, the computer systems of various third parties on which we rely, including our CROs, CMOs and other contractors, consultants and law and accounting firms, may sustain damage from computer viruses, unauthorized access, data breaches, phishing attacks, cybercriminals, natural disasters (including hurricanes and earthquakes), terrorism, war and telecommunication and electrical failures. We rely on our third-party providers to implement effective security measures and identify and correct for any such failures, deficiencies or breaches.

Our employees, principal investigators, CROs, CMOs and consultants may engage in misconduct or other improper activities, including non-compliance with regulatory standards and requirements and insider trading.

We are exposed to the risk of fraud or other misconduct by our employees, principal investigators, consultants and commercial partners. Misconduct by these parties could include intentional failures to comply with the regulations of FDA and non-U.S. regulators, provide accurate information to the FDA and non-U.S. regulators, comply with healthcare fraud and abuse laws and regulations in the United States and abroad, report financial information or data accurately or disclose unauthorized activities to us. In particular, sales, marketing and business arrangements in the healthcare industry are subject to extensive laws and regulations intended to prevent fraud, misconduct, kickbacks, self-dealing and other abusive practices. These laws and regulations may restrict or prohibit a wide range of pricing, discounting, marketing and promotion, sales commission, customer incentive programs and other business arrangements. Such misconduct could also involve the improper use of information obtained in the course of clinical studies, which could result in regulatory sanctions and cause serious harm to our reputation. We have adopted a code of conduct applicable to all of our employees, but it is not always possible to identify and deter employee misconduct, and the precautions we take to detect and prevent this activity may not be effective in controlling unknown or unmanaged risks or losses or in protecting us from governmental investigations or other actions or lawsuits stemming from a failure to comply with these laws or regulations. If any such actions are instituted against us, and we are not successful in defending ourselves or asserting our rights, those actions could have a significant impact on our business, including the imposition of significant fines or other sanctions.

Our business entails a significant risk of product liability and our ability to obtain sufficient insurance coverage could have a material and adverse effect on our business, financial condition, results of operations and prospects.

We will face an inherent risk of product liability exposure related to the testing of STK-001 and our future product candidates in clinical trials and will face an even greater risk if we commercialize any of our product candidates. If we cannot successfully defend ourselves against claims that our product candidates caused injuries, we could incur substantial liabilities. Regardless of merit or eventual outcome, liability claims may result in:

- decreased demand for any product candidates that we may develop;
- injury to our reputation and significant negative media attention;
- withdrawal of clinical trial participants;
- significant time and costs to defend the related litigation;
- substantial monetary awards to trial participants or patients;
- loss of revenue; and
- the inability to commercialize any product candidates that we may develop.

While we currently have product liability insurance that we believe is appropriate for our stage of development, we may need to obtain higher levels prior to clinical development or marketing STK-001 or any of our future product candidates. Any insurance we have or may obtain may not provide sufficient coverage against potential liabilities. Furthermore, clinical trial and product liability insurance is becoming increasingly expensive. As a result, we may be unable to obtain sufficient insurance at a reasonable cost to protect us against losses caused by product liability claims that could have a material and adverse effect on our business, financial condition, results of operations and prospects.

Risks related to our common stock and this offering

The market price of our stock may be volatile, and you could lose all or part of your investment.

The trading price of our common stock following this offering is likely to be highly volatile and subject to wide fluctuations in response to various factors, some of which we cannot control. As a result of this volatility, investors may not be able to sell their common stock at or above the initial public offering price. The market price for our common stock may be influenced by many factors, including the other risks described in this section of the prospectus entitled “Risk factors” and the following:

- results of preclinical studies and clinical trials of our product candidates, or those of our competitors or our existing or future collaborators;
- regulatory or legal developments in the United States and other countries, especially changes in laws or regulations applicable to our product candidates;
- the success of competitive products or technologies;
- introductions and announcements of new products by us, our future commercialization partners, or our competitors, and the timing of these introductions or announcements;
- actions taken by regulatory agencies with respect to our product candidates, clinical studies, manufacturing process or sales and marketing terms;
- actual or anticipated variations in our financial results or those of companies that are perceived to be similar to us;
- the success of our efforts to acquire or in-license additional technologies, products or product candidates;
- developments concerning any future collaborations, including but not limited to those with our sources of manufacturing supply and our commercialization partners;
- market conditions in the pharmaceutical and biotechnology sectors;
- announcements by us or our competitors of significant acquisitions, strategic collaborations, joint ventures or capital commitments;
- developments or disputes concerning patents or other proprietary rights, including patents, litigation matters and our ability to obtain patent protection for our product candidates and products;
- our ability or inability to raise additional capital and the terms on which we raise it;
- the recruitment or departure of key personnel;
- changes in the structure of healthcare payment systems;
- actual or anticipated changes in earnings estimates or changes in stock market analyst recommendations regarding our common stock, other comparable companies or our industry generally;

- our failure or the failure of our competitors to meet analysts' projections or guidance that we or our competitors may give to the market;
- fluctuations in the valuation of companies perceived by investors to be comparable to us;
- announcement and expectation of additional financing efforts;
- speculation in the press or investment community;
- trading volume of our common stock;
- sales of our common stock by us or our stockholders;
- the concentrated ownership of our common stock;
- changes in accounting principles;
- terrorist acts, acts of war or periods of widespread civil unrest;
- natural disasters and other calamities; and
- general economic, industry and market conditions.

In addition, the stock market in general, and the markets for pharmaceutical, biopharmaceutical and biotechnology stocks in particular, have experienced extreme price and volume fluctuations that have been often unrelated or disproportionate to the operating performance of the issuer. These broad market and industry factors may seriously harm the market price of our common stock, regardless of our actual operating performance. The realization of any of the above risks or any of a broad range of other risks, including those described in this "Risk factors" section, could have a dramatic and adverse impact on the market price of our common stock.

You will experience immediate and substantial dilution as a result of this offering and may experience additional dilution in the future.

If you purchase common stock in this offering, assuming an initial public offering price of $15.00 per share, the midpoint of the estimated price range set forth on the cover of this prospectus, you will incur immediate and substantial dilution of $8.74 per share, representing the difference between the assumed initial public offering price of $15.00 share and our pro forma net tangible book value per share as of March 31, 2019 after giving effect to this offering and the conversion of all outstanding shares of our redeemable convertible preferred stock upon the completion of this offering.

Moreover, we issued options in the past to acquire common stock at prices below the assumed initial public offering price. As of March 31, 2019, there were 4,034,649 shares of common stock subject to outstanding options under our 2014 Equity Incentive Plan. To the extent that these outstanding options and options granted in the future are ultimately exercised, you will incur further dilution.

An active and liquid trading market for our common stock may not develop and you may not be able to resell your shares of common stock at or above the public offering price.

Prior to this offering, no market for shares of our common stock existed and an active trading market for our shares may never develop or be sustained following this offering. The initial public offering price for our common stock will be determined through negotiations with the underwriters and the negotiated price may not be indicative of the market price of our common stock after this offering. The market value of our common stock may decrease from the initial public offering price. As a result of these and other factors, you may be

unable to resell your shares of our common stock at or above the initial public offering price. The lack of an active market may impair your ability to sell your shares at the time you wish to sell them or at a price that you consider reasonable. The lack of an active market may also reduce the fair market value of your shares. Furthermore, an inactive market may also impair our ability to raise capital by selling shares of our common stock and may impair our ability to enter into strategic collaborations or acquire companies or products by using our shares of common stock as consideration. Further, Apple Tree Partners IV, L.P, together with its affiliates, or Apple Tree, owned approximately 65% of our outstanding capital stock as of March 31, 2019, and the sales of stock by Apple Tree, or the lack thereof, may have a material adverse effect on our stock price and trading volume.

Our principal stockholders and management own a significant percentage of our stock and will be able to exert significant control over matters subject to stockholder approval.

Based on the beneficial ownership of our common stock as of March 31, 2019, prior to this offering, our executive officers, directors and affiliates beneficially owned approximately 68.4% of our voting stock and, upon the completion of this offering, that same group will hold approximately 53.7% of our outstanding voting stock (assuming no exercise of the underwriters' option to purchase additional shares, no exercise of outstanding options and no purchases of shares in this offering by any of this group), in each case assuming the conversion of all outstanding shares of our redeemable convertible preferred stock into shares of our common stock. As a result, these stockholders, if acting together, will continue to have significant influence over the outcome of corporate actions requiring stockholder approval, including the election of directors, amendment of our organizational documents, any merger, consolidation or sale of all or substantially all of our assets and any other significant corporate transaction. The interests of these stockholders may not be the same as or may even conflict with your interests. For example, these stockholders could delay or prevent a change of control of our company, even if such a change of control would benefit our other stockholders, which could deprive our stockholders of an opportunity to receive a premium for their common stock as part of a sale of our company or our assets and might affect the prevailing market price of our common stock. The significant concentration of stock ownership may adversely affect the trading price of our common stock due to investors' perception that conflicts of interest may exist or arise.

Upon the closing of this offering, we will qualify as a "controlled company" within the meaning of the Nasdaq listing rules and will qualify for exemptions from certain corporate governance requirements. While we do not intend to rely on these exemptions, we may change our decision in the future.

After the completion of this offering, an entity affiliated with Apple Tree Partners will beneficially own a majority of the voting power of all outstanding shares of our common stock. As a result, after the filing of our restated certificate of incorporation and the automatic termination of the amended and restated voting agreement as of the closing of this offering, we will be a "controlled company" within the meaning of the corporate governance standards of Nasdaq. Under these rules, a "controlled company" may elect not to comply with certain corporate governance requirements, including:

- the requirement that a majority of our board of directors consists of independent directors;
- the requirement that we have a nominating and corporate governance committee that is composed entirely of independent directors with a written charter addressing the committee's purpose and responsibilities;
- the requirement that we have a compensation committee that is composed entirely of independent directors with a written charter addressing the committee's purpose and responsibilities; and
- the requirement for an annual performance evaluation of the nominating and corporate governance and compensation committees.

Although we do not intend to take advantage of these exemptions, we could change our decision in the future. In such event, you would not have the same protections afforded to shareholders of companies that are subject to all of the Nasdaq corporate governance requirements.

A sale of a substantial number of shares of our common stock may cause the price of our common stock to decline.

Based on shares outstanding as of March 31, 2019, upon completion of this offering, we will have outstanding a total of 30,269,808 shares of common stock. Of these shares, only 6,700,000 shares of common stock sold in this offering, or 7,705,000 shares if the underwriters exercise their option to purchase additional shares in full, will be freely tradable, without restriction, in the public market immediately after this offering. Each of our officers, directors and substantially all of our stockholders have entered or will enter into lock-up agreements with the underwriters that restrict their ability to sell or transfer their shares. The lock-up agreements pertaining to this offering will expire 180 days from the date of this prospectus. However, our underwriters may, in their sole discretion, permit our officers, directors and other current stockholders who are subject to the contractual lock-up to sell shares prior to the expiration of the lock-up agreements. After the lock-up agreements expire, based on shares outstanding as of March 31, 2019, up to an additional 23,569,808 shares of common stock will be eligible for sale in the public market, 16,771,126 of which are held by our officers, directors and their affiliated entities, and will be subject to volume limitations under Rule 144 under the Securities Act. In addition, 4,034,649 shares of our common stock that are subject to outstanding options as of March 31, 2019 will become eligible for sale in the public market to the extent permitted by the provisions of various vesting agreements, the lock-up agreements and Rules 144 and 701 under the Securities Act.

After this offering, the holders of an aggregate of 22,677,585 shares of our outstanding common stock as of March 31, 2019 will have rights, subject to some conditions, to require us to file registration statements covering their shares or to include their shares in registration statements that we may file for ourselves or our stockholders. We also intend to register shares of common stock that we may issue under our equity incentive plans. Once we register these shares, they will be able to be sold freely in the public market upon issuance, subject to the 180-day lock-up period under the lock-up agreements described above and in the section entitled “Underwriting.”

We cannot predict what effect, if any, sales of our shares in the public market or the availability of shares for sale will have on the market price of our common stock. However, future sales of substantial amounts of our common stock in the public market, including shares issued upon exercise of outstanding options, or the perception that such sales may occur, could adversely affect the market price of our common stock.

We also expect that significant additional capital may be needed in the future to continue our planned operations. To raise capital, we may sell common stock, convertible securities or other equity securities in one or more transactions at prices and in a manner we determine from time to time. These sales, or the perception in the market that the holders of a large number of shares intend to sell shares, could reduce the market price of our common stock.

We will have broad discretion in the use of the net proceeds from this offering and may not use them effectively.

Our management will have broad discretion in the application of the net proceeds from this offering, and you will be relying on the judgment of our management regarding the application of these proceeds. You will not have the opportunity, as part of your investment decision, to assess whether we are using the proceeds appropriately. Our management might not apply our net proceeds in ways that ultimately increase the value of your investment. If we do not invest or apply the net proceeds from this offering in ways that enhance

stockholder value, we may fail to achieve expected financial results, which could cause our stock price to decline.

If securities or industry analysts do not publish research or reports about our business, or if they issue an adverse or misleading opinion regarding our stock, our stock price and trading volume could decline.

The trading market for our common stock will be influenced by the research and reports that industry or securities analysts publish about us or our business. We do not currently have and may never obtain research coverage by securities and industry analysts. If no or few securities or industry analysts commence coverage of us, the trading price for our common stock could be impacted negatively. In the event we obtain securities or industry analyst coverage, if any of the analysts who cover us issue an adverse or misleading opinion regarding us, our business model, our intellectual property or our stock performance, or if our preclinical studies and clinical trials and results of operations fail to meet the expectations of analysts, our stock price would likely decline. If one or more of such analysts cease coverage of us or fail to publish reports on us regularly, we could lose visibility in the financial markets, which in turn could cause a decline in our stock price or trading volume.

The future sale and issuance of equity or of debt securities that are convertible into equity will dilute our share capital.

We may choose to raise additional capital in the future, depending on market conditions, strategic considerations and operational requirements. To the extent that additional capital is raised through the sale and issuance of shares or other securities convertible into shares, our stockholders will be diluted. Future issuances of our common stock or other equity securities, or the perception that such sales may occur, could adversely affect the trading price of our common stock and impair our ability to raise capital through future offerings of shares or equity securities. No prediction can be made as to the effect, if any, that future sales of common stock or the availability of common stock for future sales will have on the trading price of our common stock.

We are an "emerging growth company" and a "smaller reporting company" and we cannot be certain if the reduced reporting requirements applicable to emerging growth companies or smaller reporting companies will make our common stock less attractive to investors.

We are an "emerging growth company" as defined in the Jumpstart Our Business Startups Act of 2012, or the JOBS Act. For as long as we continue to be an emerging growth company, we may take advantage of exemptions from various reporting requirements that are applicable to other public companies that are not emerging growth companies, including (i) not being required to comply with the auditor attestation requirements of Section 404 of the Sarbanes-Oxley Act of 2002, as amended, or the Sarbanes-Oxley Act, (ii) reduced disclosure obligations regarding executive compensation in this prospectus and our periodic reports and proxy statements and (iii) exemptions from the requirements of holding nonbinding advisory stockholder votes on executive compensation and stockholder approval of any golden parachute payments not approved previously. In addition, as an emerging growth company, we are only required to provide two years of audited financial statements and two years of selected financial data in this prospectus.

We could be an emerging growth company for up to five years following the completion of this offering, although circumstances could cause us to lose that status earlier, including if we are deemed to be a "large accelerated filer," which occurs when the market value of our common stock that is held by non-affiliates exceeds $700 million as of the prior June 30, or if we have total annual gross revenue of $1.07 billion or more during any fiscal year before that time, in which cases we would no longer be an emerging growth company as of the following December 31, or if we issue more than $1.0 billion in non-convertible debt during the prior three-year period before that time, in which case we would no longer be an emerging growth company immediately. Even after we no longer qualify as an emerging growth company, we may still qualify as a "smaller

reporting company," as such term is defined in Rule 12b-2 under the Securities Exchange Act of 1934, as amended, the Exchange Act, which would allow us to take advantage of many of the same exemptions from disclosure requirements, including not being required to comply with the auditor attestation requirements of Section 404 of the Sarbanes-Oxley Act and reduced disclosure obligations regarding executive compensation in this prospectus and in our periodic reports and proxy statements. We cannot predict if investors will find our common stock less attractive because we may rely on these exemptions. If some investors find our common stock less attractive as a result, there may be a less active trading market for our common stock and our share price may be more volatile.

Under the JOBS Act, emerging growth companies can also delay adopting new or revised accounting standards until such time as those standards apply to private companies. We have elected to take advantage of the benefits of this extended transition period. Our consolidated financial statements may therefore not be comparable to those of companies that comply with such new or revised accounting standards. Until the date that we are no longer an "emerging growth company" or affirmatively and irrevocably opt out of the exemption provided by Section 7(a)(2)(B) of the Securities Act, upon issuance of a new or revised accounting standard that applies to our consolidated financial statements and that has a different effective date for public and private companies, we will disclose the date on which adoption is required for non-emerging growth companies and the date on which we will adopt the recently issued accounting standard.

We are also a "smaller reporting company," meaning that the market value of our stock held by non-affiliates plus the proposed aggregate amount of gross proceeds to us as a result of this offering is less than $700 million and our annual revenue is less than $100 million during the most recently completed fiscal year. We may continue to be a smaller reporting company after this offering if either (i) the market value of our stock held by non-affiliates is less than $250 million or (ii) our annual revenue is less than $100 million during the most recently completed fiscal year and the market value of our stock held by non-affiliates is less than $700 million. If we are a smaller reporting company at the time we cease to be an emerging growth company, we may continue to rely on exemptions from certain disclosure requirements that are available to smaller reporting companies. Specifically, as a smaller reporting company we may choose to present only the two most recent fiscal years of audited financial statements in our Annual Report on Form 10-K and, similar to emerging growth companies, smaller reporting companies have reduced disclosure obligations regarding executive compensation.

Anti-takeover provisions in our charter documents and under Delaware law could make an acquisition of us, which may be beneficial to our stockholders, more difficult and may prevent attempts by our stockholders to replace or remove our current management.

Our restated certificate of incorporation and our restated bylaws that will be in effect upon completion of this offering contain provisions that could delay or prevent a change in control of our company. These provisions could also make it difficult for stockholders to elect directors who are not nominated by current members of our board of directors or take other corporate actions, including effecting changes in our management. These provisions:

- establish a classified board of directors so that not all members of our board are elected at one time;
- permit only the board of directors to establish the number of directors and fill vacancies on the board;
- provide that directors may only be removed "for cause" and only with the approval of two-thirds of our stockholders;
- require super-majority voting to amend some provisions in our restated certificate of incorporation and restated bylaws;

- authorize the issuance of “blank check” preferred stock that our board could use to implement a stockholder rights plan;
- eliminate the ability of our stockholders to call special meetings of stockholders;
- prohibit stockholder action by written consent, which requires all stockholder actions to be taken at a meeting of our stockholders;
- prohibit cumulative voting; and
- establish advance notice requirements for nominations for election to our board or for proposing matters that can be acted upon by stockholders at annual stockholder meetings.

In addition, our restated certificate of incorporation, to the fullest extent permitted by law, will provide that the Court of Chancery of the State of Delaware will be the exclusive forum for: any derivative action or proceeding brought on our behalf; any action asserting a breach of fiduciary duty; any action asserting a claim against us arising pursuant to the Delaware General Corporation Law, or the DGCL, our restated certificate of incorporation, or our restated bylaws; or any action asserting a claim against us that is governed by the internal affairs doctrine. This exclusive forum provision does not apply to suits brought to enforce a duty or liability created by the Exchange Act. It could apply, however, to a suit that falls within one or more of the categories enumerated in the exclusive forum provision and asserts claims under the Securities Act, inasmuch as Section 22 of the Securities Act creates concurrent jurisdiction for federal and state courts over all suits brought to enforce any duty or liability created by the Securities Act or the rule and regulations thereunder. There is uncertainty as to whether a court would enforce such provision with respect to claims under the Securities Act, and our stockholders will not be deemed to have waived our compliance with the federal securities laws and the rules and regulations thereunder.

This choice of forum provision may limit a stockholder’s ability to bring a claim in a judicial forum that it finds favorable for disputes with us or any of our directors, officers, or other employees, which may discourage lawsuits with respect to such claims. Alternatively, if a court were to find the choice of forum provisions contained in our restated certificate of incorporation to be inapplicable or unenforceable in an action, we may incur additional costs associated with resolving such action in other jurisdictions, which could harm our business, results of operations and financial condition.

In addition, Section 203 of the DGCL may discourage, delay or prevent a change in control of our company. Section 203 imposes certain restrictions on mergers, business combinations and other transactions between us and holders of 15% or more of our common stock.

We will incur increased costs as a result of operating as a public company, and our management will be required to devote substantial time to new compliance initiatives and corporate governance practices.

As a public company, and particularly after we are no longer an emerging growth company, we will incur significant legal, accounting and other expenses that we did not incur as a private company. The Sarbanes-Oxley Act, the Dodd-Frank Wall Street Reform and Consumer Protection Act, the listing requirements of the Nasdaq Global Market, or Nasdaq, and other applicable securities rules and regulations impose various requirements on public companies, including establishment and maintenance of effective disclosure and financial controls and corporate governance practices. Our management and other personnel will need to devote a substantial amount of time to these compliance initiatives. Moreover, we expect these rules and regulations to substantially increase our legal and financial compliance costs and to make some activities more time consuming and costly. For example, we expect that these rules and regulations may make it more difficult and more expensive for us to obtain director and officer liability insurance and we may be required to incur

substantial costs to maintain sufficient coverage. We cannot predict or estimate the amount or timing of additional costs we may incur to respond to these requirements. The impact of these requirements could also make it more difficult for us to attract and retain qualified persons to serve on our board of directors, our board committees or as executive officers. Moreover, these rules and regulations are often subject to varying interpretations, in many cases due to their lack of specificity, and, as a result, their application in practice may evolve over time as new guidance is provided by regulatory and governing bodies. This could result in continuing uncertainty regarding compliance matters and higher costs necessitated by ongoing revisions to disclosure and governance practices.

If we fail to maintain proper and effective internal control over financial reporting in the future, our ability to produce accurate and timely financial statements could be impaired, which could harm our operating results, investors' views of us and, as a result, the value of our common stock.

We are not currently required to comply with the Securities and Exchange Commission's, or SEC's, rules that implement Section 404 of the Sarbanes-Oxley Act, and are therefore not required to make a formal assessment of the effectiveness of our internal control over financial reporting for that purpose. Pursuant to Section 404, we will be required to furnish a report by our management on our internal control over financial reporting. However, while we remain an emerging growth company, we will not be required to include an attestation report on internal control over financial reporting issued by our independent registered public accounting firm. To achieve compliance with Section 404 within the prescribed period, we will be engaged in a process to document and evaluate our internal control over financial reporting, which is both costly and challenging. In this regard, we will need to continue to dedicate internal resources, potentially engage outside consultants and adopt a detailed work plan to assess and document the adequacy of internal control over financial reporting, continue steps to improve control processes as appropriate, validate through testing that controls are functioning as documented and implement a continuous reporting and improvement process for internal control over financial reporting. Despite our efforts, there is a risk that we will not be able to conclude, within the prescribed timeframe or at all, that our internal control over financial reporting is effective as required by Section 404. If we identify one or more material weaknesses, it could result in an adverse reaction in the financial markets due to a loss of confidence in the reliability of our consolidated financial statements. In addition, if we are not able to continue to meet these requirements, we may not be able to remain listed on Nasdaq.

As we grow, we expect to hire additional personnel and may utilize external temporary resources to implement, document and modify policies and procedures to maintain effective internal controls. However, it is possible that we may identify deficiencies and weaknesses in our internal controls. If material weaknesses or deficiencies in our internal controls exist and go undetected or unremediated, our consolidated financial statements could contain material misstatements that, when discovered in the future, could cause us to fail to meet our future reporting obligations and cause the price of our common stock to decline.

Because we do not anticipate paying any cash dividends on our capital stock in the foreseeable future, capital appreciation, if any, will be your sole source of gain.

We have never declared or paid cash dividends on our capital stock. We currently intend to retain all of our future earnings, if any, to finance the growth and development of our business. As a result, capital appreciation, if any, of our common stock will be your sole source of gain for the foreseeable future.

We may be subject to securities litigation, which is expensive and could divert management attention.

The market price of our common stock may be volatile and, in the past, companies that have experienced volatility in the market price of their stock have been subject to securities class action litigation. We may be the target of this type of litigation in the future. Securities litigation against us could result in substantial costs and divert our management's attention from other business concerns, which could seriously harm our business.

Special note regarding forward-looking statements

This prospectus, including the sections entitled “Prospectus summary,” “Risk factors,” “Use of proceeds,” “Management’s discussion and analysis of financial condition and results of operations,” and “Business” contains forward-looking statements. The words “believe,” “may,” “will,” “potentially,” “estimate,” “continue,” “anticipate,” “intend,” “could,” “would,” “project,” “plan,” “expect” and similar expressions that convey uncertainty of future events or outcomes are intended to identify forward-looking statements. These forward-looking statements are subject to a number of risks, uncertainties and assumptions, including those described in “Risk factors” and elsewhere in this prospectus. Moreover, we operate in a competitive and rapidly changing environment, and new risks emerge from time to time. It is not possible for our management to predict all risks, nor can we assess the impact of all factors on our business or the extent to which any factor, or combination of factors, may cause actual results to differ materially from those contained in any forward-looking statements we may make. In light of these risks, uncertainties and assumptions, the forward-looking events and circumstances discussed in this prospectus may not occur and actual results could differ materially and adversely from those anticipated or implied in the forward-looking statements. Forward-looking statements include statements about:

- our ability to develop, obtain regulatory approval for and commercialize STK-001 and our future product candidates;
- our success in early preclinical studies or clinical trials, which may not be indicative of results obtained in later studies or trials;
- our ability to obtain regulatory approval to commercialize STK-001 or any other future product candidate;
- our ability to identify patients with the diseases treated by STK-001 or our future product candidates, and to enroll patients in trials;
- the success of our efforts to use TANGO to expand our pipeline of product candidates and develop marketable products;
- our ability to obtain, maintain and protect our intellectual property;
- our reliance upon intellectual property licensed from third parties;
- our ability to identify, recruit and retain key personnel;
- our use of proceeds from this offering;
- our financial performance; and
- developments or projections relating to our competitors or our industry.

You should not rely upon forward-looking statements as predictions of future events. Although we believe that the expectations reflected in the forward-looking statements are reasonable, we cannot guarantee that the future results, levels of activity, performance or events and circumstances reflected in the forward-looking statements will be achieved or occur. We undertake no obligation to update publicly any forward-looking statements for any reason after the date of this prospectus to conform these statements to actual results or to changes in our expectations, except as required by law.

You should read this prospectus and the documents that we reference in this prospectus and have filed with the SEC as exhibits to the registration statement of which this prospectus is a part with the understanding that our actual future results, levels of activity, performance and events and circumstances may be materially different from what we expect.

Use of proceeds

We estimate that the net proceeds from our sale of 6,700,000 shares of common stock in this offering at an assumed initial public offering price of $15.00 per share, which is the midpoint of the estimated price range set forth on the cover of this prospectus, after deducting the estimated underwriting discounts and commissions and estimated offering expenses, will be approximately $91.0 million. If the underwriters exercise their option to purchase additional shares in full, then the net proceeds will be approximately $105.0 million.

Each $1.00 increase (decrease) in the assumed initial public offering price of $15.00 per share, which is the midpoint of the estimated price range set forth on the cover of this prospectus, would increase (decrease) the net proceeds to us from this offering by $6.2 million, assuming the number of shares offered, as set forth on the cover of this prospectus, remains the same, and after deducting the estimated underwriting discounts and commissions. Similarly, each increase (decrease) of 1,000,000 shares in the number of shares of common stock offered would increase (decrease) the net proceeds that we receive from this offering by $14.0 million, assuming that the assumed initial public offering price remains the same and after deducting the estimated underwriting discounts and commissions.

We currently intend to use the net proceeds we receive from this offering as follows:

- approximately $36.0 million to $39.0 million to advance our lead product candidate, STK-001, through initiation of a Phase 3 clinical trial;
- approximately $34.0 million to $37.0 million to nominate, conduct preclinical studies for and demonstrate clinical proof of concept for additional product candidates; and
- any remaining amounts to fund working capital and general corporate purposes.

Based on our planned use of the net proceeds, we estimate such funds, together with our existing cash, cash equivalents and restricted cash, will be sufficient for us to fund our operating expenses and capital expenditure requirements through the end of 2022.

The expected use of the net proceeds from the offering represents our intentions based upon our current plans and business conditions. The amounts we actually expend in these areas, and the timing thereof, may vary significantly from our current intentions and will depend on a number of factors, including the success of research and product development efforts, cash generated from future operations and actual expenses to operate our business. We may use a portion of the net proceeds for the acquisition of, or investment in, businesses that complement our business, although we have no present commitments or agreements.

The amounts and timing of our clinical expenditures and the extent of clinical development may vary significantly depending on numerous factors, including the status, results and timing of our current preclinical studies and those clinical trials which we may commence in the future, the product approval process with the FDA and other regulatory agencies, our current collaborations and any new collaborations we may enter into with third parties and any unforeseen cash needs. As a result, we cannot predict with any certainty all of the particular uses for the net proceeds or the amounts that we will actually spend on the uses set forth above. Accordingly, our management will have broad discretion in the application of the net proceeds, and investors will be relying on the judgment of our management regarding the application of the net proceeds of this offering.

The expected net proceeds of this offering will not be sufficient for us to fund any of our product candidates through regulatory approval, and we will need to raise substantial additional capital to complete the development and commercialization of our product candidates.

Pending the uses described above, we intend to invest the net proceeds from this offering in short term, investment-grade interest-bearing securities such as money market accounts, certificates of deposit, commercial paper and guaranteed obligations of the U.S. government.

Dividend policy

We have never declared or paid cash dividends on our common stock. We currently intend to retain all available funds and any future earnings for use in the operation of our business and do not anticipate paying any cash dividends on our common stock in the foreseeable future. Any future determination to declare dividends will be made at the discretion of our board of directors and will depend on our financial condition, operating results, capital requirements, general business conditions and other factors that our board of directors may deem relevant.

Capitalization

The following table sets forth our cash, cash equivalents and restricted cash and capitalization as of March 31, 2019 on:

- an actual basis;
- a pro forma basis, giving effect to (i) the automatic conversion of 22,677,585 outstanding shares of our convertible preferred stock as of March 31, 2019 into an aggregate of 22,677,585 shares of common stock immediately prior to the completion of this offering and (ii) the effectiveness of our restated certificate of incorporation in connection with the completion of this offering; and
- a pro forma as adjusted basis, giving effect to (i) the pro forma adjustments described above and (ii) the sale of 6,700,000 shares of common stock in this offering, based upon an assumed initial public offering price of $15.00 per share, which is the midpoint of the estimated price range set forth on the cover of this prospectus, after deducting the estimated underwriting discounts and commissions and estimated offering expenses.

The pro forma as adjusted information set forth in the table below is illustrative only and will be adjusted based on the actual initial public offering price and other terms of this offering as determined at pricing.

You should read this table together with “Management’s discussion and analysis of financial condition and results of operations,” “Selected consolidated financial data” and our audited consolidated financial statements and related notes, each included elsewhere in this prospectus.

	March 31, 2019		
	Actual	Pro forma	Pro forma as adjusted[1]
			(Unaudited)
			(in thousands, except share and per share amounts)
Cash, cash equivalents and restricted cash	$ 98,913	$ 98,913	$ 190,070
Convertible preferred stock, par value of $0.0001 per share; 225,584,874 shares authorized, 22,677,585 shares issued and outstanding as of March 31, 2019 and December 31, 2018; aggregate liquidation preference of $130,850 at March 31, 2019 (unaudited) and December 31, 2018	2	—	—
Preferred stock, $0.0001 par value: no shares authorized, issued or outstanding, actual; 10,000,000 shares authorized, no shares issued or outstanding pro forma and pro forma as adjusted	—	—	—
Common stock, $0.0001 par value: 278,527,249 shares authorized; 892,223 shares issued and outstanding, actual; 300,000,000 shares authorized, 23,569,808 shares issued and outstanding, pro forma, 30,269,808 shares issued and outstanding, pro forma as adjusted	—	2	3
Additional paid-in-capital	131,031	131,031	222,045
Accumulated deficit	(31,452)	(31,452)	(31,452)
Total stockholders’ equity	99,581	99,581	190,596
Total capitalization	$ 99,581	$ 99,581	$ 190,596

(1) Each $1.00 increase (decrease) in the assumed initial public offering price of $15.00 per share, which is the midpoint of the estimated price range set forth on the cover of this prospectus, would increase (decrease) each of our pro forma as adjusted cash, cash equivalents and restricted cash, additional paid-in-capital, total stockholders’ equity and total capitalization by approximately $6.2 million, assuming that the number of shares offered remains the same and after deducting the estimated underwriting discounts and commissions. Similarly, each increase (decrease) of 1,000,000 shares in the number of shares of common stock offered would increase (decrease) each of our pro forma as adjusted cash, cash equivalents and restricted cash, additional paid-in-capital, total stockholders’ equity and total capitalization by approximately $14.0 million, assuming the assumed initial public offering price remains the same and after deducting the estimated underwriting discounts and commissions.

The table above excludes the following shares:

- 4,034,649 shares of common stock issuable upon the exercise of options outstanding as of March 31, 2019 under our 2014 Equity Incentive Plan, or the 2014 Plan, with a weighted-average exercise price of $1.64 per share;
- 191,372 shares of common stock issuable upon the exercise of options granted after March 31, 2019 under the 2014 Plan, with a weighted-average exercise price of $9.07 per share; and
- 2,910,316 shares of common stock reserved for future issuance under our stock-based compensation plans, consisting of (i) 395,316 shares of common stock reserved for future issuance under our 2014 Plan as of March 31, 2019, (ii) 2,200,000 shares of common stock reserved for future issuance under our 2019 Equity Incentive Plan, which will become effective on the date immediately prior to the date of the effectiveness of the registration statement of which this prospectus forms a part and (iii) 315,000 shares of common stock reserved for future issuance under our 2019 Employee Stock Purchase Plan, which will become effective on the date of the effectiveness of the registration statement of which this prospectus forms a part. Upon completion of this offering, any remaining shares available for issuance under our 2014 Plan will be added to the shares reserved under our 2019 Equity Incentive Plan and we will cease granting awards under our 2014 Plan. Our 2019 Equity Incentive Plan and 2019 Employee Stock Purchase Plan also provide for automatic annual increases in the number of shares reserved under the plans each year, as more fully described in “Executive compensation—Equity compensation plans and other benefit plans.”

Dilution

If you invest in our common stock in this offering, your ownership interest will be immediately diluted to the extent of the difference between the amount per share paid by purchasers of shares of common stock in this offering and the pro forma as adjusted net tangible book value per share of common stock immediately after this offering.

Net tangible book value (deficit) per share is determined by dividing our total tangible assets (which excludes deferred offering costs) less our total liabilities and convertible preferred stock by the number of shares of common stock outstanding. Our historical net tangible book value (deficit) as of March 31, 2019 was $(31.9) million, or $(35.70) per share, based on 892,223 shares of common stock outstanding as of March 31, 2019. Our pro forma net tangible book value as of March 31, 2019 was approximately $98.5 million, or $4.18 per share of common stock. Our pro forma net tangible book value per share represents the amount of our total tangible assets (which excludes deferred offering costs) reduced by the amount of our total liabilities and divided by the total number of shares of our common stock outstanding as of March 31, 2019, after giving effect to the automatic conversion of 22,677,585 outstanding shares of our convertible preferred stock as of March 31, 2019 into an aggregate of 22,677,585 shares of common stock immediately prior to the completion of this offering.

Net tangible book value dilution per share to new investors in this offering represents the difference between the amount per share paid by purchasers of shares of common stock in this offering and the pro forma as adjusted net tangible book value per share of common stock immediately after completion of this offering. After giving effect to (i) the pro forma adjustments set forth above and (ii) our sale in this offering of 6,700,000 shares of our common stock at an assumed initial public offering price of $15.00 per share, which is the midpoint of the estimated price range set forth on the cover of this prospectus, and after deducting the estimated underwriting discounts and commissions and estimated offering expenses, our pro forma as adjusted net tangible book value as of March 31, 2019 would have been approximately $189.5 million, or $6.26 per share of our common stock. This represents an immediate increase in pro forma net tangible book value of $2.08 per share to our existing stockholders and an immediate dilution of $8.74 per share to investors in this offering, as illustrated in the following table:

Assumed initial public offering price, per share		$15.00
Pro forma net tangible book value per share as of March 31, 2019	$4.18	
Increase in pro forma net tangible book value per share attributable to new investors in this offering	2.08	
Pro forma as adjusted net tangible book value per share after this offering		6.26
Dilution per share to new investors in this offering		$ 8.74

Each $1.00 increase (decrease) in the assumed initial public offering price of $15.00 per share, which is the midpoint of the estimated price range set forth on the cover of this prospectus, would increase (decrease) our pro forma as adjusted net tangible book value by $6.2 million, or $0.21 per share and the dilution in pro forma as adjusted net tangible book value per share to new investors in this offering by $0.79 per share, assuming the number of shares offered, as set forth on the cover of this prospectus, remains the same, and after deducting the estimated underwriting discounts and commissions. Similarly, each increase of 1,000,000 shares in the number of shares of common stock offered in this offering would increase our pro forma as adjusted net tangible book value by approximately $14.0 million, or approximately $0.25 per share, and would increase dilution per share to new investors in this offering by approximately $0.25 per share and each decrease of 1,000,000 shares in the number of shares of common stock offered in this offering would decrease our pro

forma as adjusted net tangible book value by approximately $14.0 million, or approximately $0.26 per share, and would decrease dilution per share to new investors in this offering by approximately $0.26 per share, assuming the assumed initial public offering price per share remains the same and after deducting the estimated underwriting discounts and commissions. The pro forma as adjusted information is illustrative only, and we will adjust this information based on the actual initial public offering price and other terms of this offering determined at pricing.

If the underwriters exercise their option in full to purchase additional shares, the pro forma as adjusted net tangible book value per share after this offering would be $6.51 per share, the increase in pro forma as adjusted net tangible book value per share to existing stockholders would be $0.25 per share and the dilution to new investors in this offering would be $0.25 per share.

The following table shows, as of March 31, 2019, on a pro forma as adjusted basis described above, the differences between the existing stockholders and the purchasers of shares in this offering with respect to the number of shares purchased from us, the total consideration paid, which includes net proceeds received from the issuance of common and convertible preferred stock, cash received from the exercise of stock options, and the value of any stock issued for services and the average price paid per share (in thousands, except per share amounts and percentages):

	Shares purchased		Total consideration		Average price per share
	Number	Percent	Amount	Percent	
Existing stockholders	23,569,808	77.9%	$130,778,000	56.5%	$ 5.55
New public investors	6,700,000	22.1	100,500,000	43.5	$ 15.00
Total	30,269,808	100.0%	$231,278,000	100.0%	

Each $1.00 increase (decrease) in the assumed initial public offering price of $15.00 per share, which is the midpoint of the estimated price range set forth on the cover of this prospectus, would increase (decrease) total consideration paid by new investors and total consideration paid by all stockholders by approximately $6.2 million, assuming that the number of shares offered, as set forth on the cover of this prospectus, remains the same, and after deducting the estimated underwriting discounts and commissions. Similarly, each increase (decrease) of 1,000,000 shares in the number of shares of common stock offered in this offering would increase (decrease) total consideration paid by new investors and total consideration paid by all stockholders by approximately $14.0 million, assuming the assumed initial public offering price remains the same and after deducting the estimated underwriting discounts and commissions. In addition, to the extent that any outstanding options are exercised, investors in this offering will experience further dilution.

Except as otherwise indicated, the above discussion and tables assume no exercise of the underwriters' option to purchase additional shares. If the underwriters exercise their option to purchase additional shares in full, our existing stockholders would own 75.4% and our new investors would own 24.6% of the total number of shares of our common stock outstanding upon the completion of this offering.

The number of shares of common stock outstanding as of March 31, 2019 excludes:

- 4,034,649 shares of common stock issuable upon the exercise of options outstanding as of March 31, 2019 under our 2014 Equity Incentive Plan, or the 2014 Plan, with a weighted-average exercise price of $1.64 per share;
- 191,372 shares of common stock issuable upon the exercise of options granted after March 31, 2019 under the 2014 Plan, with a weighted-average exercise price of $9.07 per share; and

- 2,910,316 shares of common stock reserved for future issuance under our stock-based compensation plans, consisting of (i) 395,316 shares of common stock reserved for future issuance under our 2014 Plan as of March 31, 2019, (ii) 2,200,000 shares of common stock reserved for future issuance under our 2019 Equity Incentive Plan, which will become effective on the date immediately prior to the date of the effectiveness of the registration statement of which this prospectus forms a part and (iii) 315,000 shares of common stock reserved for future issuance under our 2019 Employee Stock Purchase Plan, which will become effective on the date of the effectiveness of the registration statement of which this prospectus forms a part. Upon completion of this offering, any remaining shares available for issuance under our 2014 Plan will be added to the shares reserved under our 2019 Equity Incentive Plan and we will cease granting awards under our 2014 Plan. Our 2019 Equity Incentive Plan and 2019 Employee Purchase Stock Plan also provide for automatic annual increases in the number of shares reserved under the plans each year, as more fully described in “Executive compensation—Equity compensation plans and other benefit plans.”

Selected consolidated financial data

The following tables set forth our selected consolidated financial data as of, and for the periods ended on, the dates indicated. The selected consolidated statements of operations data presented below for the years ended December 31, 2018 and 2017 and the selected consolidated balance sheet data as of December 31, 2018 and 2017 are derived from our audited consolidated financial statements included elsewhere in this prospectus. The selected consolidated statements of operations data presented below for the three months ended March 31, 2019 and 2018 and the selected consolidated balance sheet data as of March 31, 2019 are derived from our unaudited consolidated financial statements included elsewhere in this prospectus. The selected consolidated financial data included in this section are not intended to replace the consolidated financial statements and related notes included elsewhere in this prospectus. You should read the selected consolidated financial data together with the section entitled “Management’s discussion and analysis of financial condition and results of operations” and our consolidated financial statements and related notes included elsewhere in this prospectus. Our historical results are not necessarily indicative of the results to be expected for any other period in the future.

	Three months ended March 31,		Year ended December 31,	
	2019	2018	2018	2017
	(In thousands, except share and per share amounts)			
Consolidated statements of operations data:				
Revenue	$ —	$ —	$ —	$ —
Operating expenses:				
Research and development	4,133	1,252	8,371	3,598
General and administrative	2,189	660	4,410	1,956
Total operating expenses	6,322	1,912	12,781	5,554
Loss from operations	(6,322)	(1,912)	(12,781)	(5,554)
Other income (expense):				
Interest income	580	—	270	—
Other expense, net	—	—	(10)	(4)
Total other income (expense)	580	—	260	(4)
Net loss	$ (5,742)	$ (1,912)	$ (12,521)	$ (5,558)
Net loss per share attributable to common stockholders, basic and diluted[1]	$ (6.89)	$ (2.78)	$ (17.65)	$ (8.29)
Weighted-average common shares outstanding, basic and diluted[1]	833,469	686,985	709,336	670,090
Pro forma net loss per share, basic and diluted[1]	$ (0.24)		$ (0.98)	
Weighted-average shares used in computing pro forma net loss per share, basic and diluted[1]	23,511,054		12,784,811	

(1) See Notes 2 and 11 to our audited consolidated financial statements and Note 9 to our unaudited consolidated financial statements included elsewhere in this prospectus for a description of how we compute basic and diluted net loss per share and basic and diluted pro forma net loss per share, and the weighted-average number of shares used in the computation of these per share amounts.

	As of March 31, 2019	As of December 31, 2018	2017
			(In thousands)
Consolidated balance sheet data:			
Cash, cash equivalents and restricted cash	$ 98,913	$105,603	$ 1,797
Total assets	102,934	107,539	2,439
Working capital(1)	97,933	103,676	(1,805)
Total liabilities	3,353	2,471	3,731
Accumulated deficit	(31,452)	(25,710)	(13,189)
Total stockholders' equity (deficit)	99,581	105,068	(1,292)

(1) We define working capital as current assets less current liabilities. See our consolidated financial statements and related notes included elsewhere in this prospectus for further details regarding our current assets and current liabilities.

Management's discussion and analysis of financial condition and results of operations

You should read the following discussion and analysis of our financial condition and consolidated results of operations together with the section entitled "Selected financial data" and our consolidated financial statements and related notes appearing elsewhere in this prospectus. Some of the information contained in this discussion and analysis or set forth elsewhere in this prospectus, including information with respect to our plans and strategy for our business and related financing, includes forward-looking statements that involve risks and uncertainties. You should carefully read the section entitled "Risk factors" to gain an understanding of the important factors that could cause actual results to differ materially from our forward-looking statements.

Overview

We are pioneering a new way to treat the underlying causes of severe genetic diseases by precisely upregulating protein expression. We are developing novel antisense oligonucleotide, or ASO, medicines that target ribonucleic acid, or RNA, and modulate precursor-messenger RNA, or pre-mRNA, splicing to upregulate protein expression where needed and with appropriate specificity to near normal levels. We utilize our proprietary technology platform, Targeted Augmentation of Nuclear Gene Output, or TANGO, to design ASOs to upregulate the expression of protein by individual genes in a patient. Our approach is designed to allow us to deliver in a highly precise, durable and controlled manner disease-modifying therapies to a wide range of relevant tissues, including the central nervous system, or CNS, eye, kidney and liver. We designed our lead product candidate, STK-001, to treat Dravet syndrome, a severe and progressive genetic epilepsy. With a well-defined patient population based on routine genetic testing and learnings from recently approved drugs for the treatment of Dravet syndrome to inform the clinical and regulatory pathways, we anticipate an efficient clinical program for STK-001. We plan to submit an investigational new drug application for STK-001 by early 2020 and expect to initiate a Phase 1/2 clinical trial in the first half of 2020. We intend to nominate a second candidate to treat an additional genetic disease for preclinical development by the first half of 2020.

We were incorporated in June 2014. In July 2015 and April 2016, we entered into worldwide license agreements with Cold Spring Harbor Laboratory, or CSHL, and the University of Southampton, respectively, with respect to certain licensed patents and applications relating to TANGO. TANGO exploits non-productive splicing events to effect targeted enhancement of protein expression. Since our inception through March 31, 2019, our operations have been financed by net proceeds of $131.0 million primarily from the sale of convertible notes payable and our convertible preferred stock. As of December 31, 2018 and March 31, 2019, we had $105.6 million and $98.9 million, respectively, in cash, cash equivalents and restricted cash.

Since inception, we have had operating losses, the majority of which are attributable to research and development activities. Our net losses were $12.5 million and $5.6 million for the years ended December 31, 2018 and 2017 and for the three months ended March 31, 2019 and 2018 were $5.7 million and $1.9 million respectively, and as of March 31, 2019, we had an accumulated deficit of $31.5 million. Our primary use of cash is to fund operating expenses, which consist primarily of research and development expenditures, and to a lesser extent, general and administrative expenditures. Cash used to fund operating expenses is impacted by the timing of when we pay these expenses, as reflected in the change in our outstanding accounts payable and accrued expenses. We expect to continue to incur net losses for the foreseeable future, and we expect our research and development expenses, general and administrative expenses, and capital expenditures will continue to increase. In particular, we expect our expenses and losses to increase as we continue our development of, and seek regulatory approvals for, our product candidates, and begin to commercialize any approved products, as well as hire additional personnel, develop commercial infrastructure, pay fees to outside consultants, lawyers and accountants, and incur increased costs associated with being a public company such

as expenses related to services associated with maintaining compliance with Nasdaq listing rules and SEC requirements, insurance and investor relations costs. Our net losses may fluctuate significantly from quarter-to-quarter and year-to-year, depending on the timing of our clinical trials and our expenditures on other research and development activities.

Based upon our current operating plan, we believe that the net proceeds from this offering, together with our existing cash, cash equivalents and restricted cash as of March 31, 2019, will enable us to fund our operating expenses and capital expenditure requirements through the end of 2022. To date, we have not had any products approved for sale and have not generated any product sales. We do not expect to generate any revenues from product sales unless and until we successfully complete development and obtain regulatory approval for one or more of our product candidates, which we expect will take a number of years. If we obtain regulatory approval for any of our product candidates, we expect to incur significant commercialization expenses related to product sales, marketing, manufacturing and distribution. As a result, until such time, if ever, as we can generate substantial product revenue, we expect to finance our cash needs through equity offerings, debt financings or other capital sources, including potentially collaborations, licenses and other similar arrangements. However, we may be unable to raise additional funds or enter into such other arrangements when needed on favorable terms or at all. Any failure to raise capital as and when needed could have a negative impact on our financial condition and on our ability to pursue our business plans and strategies. If we are unable to raise capital, we will need to delay, reduce or terminate planned activities to reduce costs.

License agreements

Cold Spring Harbor Laboratory

In July 2015, we entered into a worldwide license agreement with CSHL, or the CSHL Agreement, with respect to the TANGO patents. Under the CSHL Agreement, we receive an exclusive (except with respect to certain government rights and non-exclusive licenses), worldwide license under certain patents and applications relating to TANGO. As part of the CSHL Agreement, we granted CSHL 164,927 shares of common stock. The CSHL Agreement obligates us to make additional payments that are contingent upon certain milestones being achieved as well as royalties on future product sales. These royalty obligations last on a product-by-product and country-by-country basis until the latest of (i) the expiration of the last valid claim of a patent covering a subject product or (ii) the expiration of any regulatory exclusivity for the subject product in a country. In addition, if we sublicense rights under the CSHL Agreement, we are required to pay a percentage of the sublicense revenue to CSHL, which may be reduced upon achievement of certain milestones for the applicable subject product. The maximum aggregate potential milestone payments payable total approximately $900,000. Additionally, certain licenses under the CSHL Agreement require us to reimburse CSHL for certain past and ongoing patent related expenses, however there were no expenses related to these reimbursable patent costs during the years ended December 31, 2018 and 2017 or for the three months ended March 31, 2019 and 2018. For more information, please see “Business—License agreements.”

University of Southampton

In April 2016, we entered into an exclusive, worldwide license agreement with the University of Southampton, or the Southampton Agreement, whereby we acquired rights to foundational technologies related to our TANGO technology. Under the Southampton Agreement, we receive an exclusive, worldwide license under certain licensed patents and applications relating to TANGO. As part of the Southampton Agreement, we paid 55,000 pounds sterling (approximately $72,000 as of the date thereof) as an up-front license fee. Under the Southampton Agreement, we may be obligated to make additional payments that are contingent upon certain milestones being achieved, as well as royalties on future product sales. These royalty obligations survive until the latest of (i) the expiration of the last valid claim of a licensed patent covering a subject product or (ii) the

expiration of any regulatory exclusivity for the subject product in a country. In addition, if we sublicense our rights under the Southampton Agreement, we are required to pay a percentage of the sublicense revenue to the University of Southampton. The maximum aggregate potential milestone payments payable by us total approximately 400,000 pounds sterling (approximately $518,000 as of March 31, 2019). As of March 31, 2019, we have recorded no liabilities under the Southampton Agreement. For more information, please see “Business—License agreements.”

Financial operations overview

Revenue

We currently do not have any products approved for sale and have not generated any revenue since inception. If we are able to successfully develop, receive regulatory approval for and commercialize any of our current or future product candidates alone or in collaboration with third parties, we may generate revenue from the sales of these product candidates.

Operating expenses

Research and development

Research and development expenses consist primarily of costs incurred for the development of our discovery work and preclinical programs, which include:

- personnel costs, which include salaries, benefits and stock-based compensation expense;
- expenses incurred under agreements with consultants, third-party contract organizations that conduct research and development activities on our behalf, costs related to production of preclinical material and laboratory and vendor expenses related to the execution of preclinical studies;
- scientific consulting, collaboration and licensing fees;
- laboratory equipment and supplies; and
- facilities costs, depreciation and other expenses related to internal research and development activities.

We use our personnel and infrastructure resources across multiple research and development programs directed toward identifying and developing product candidates. Our direct research and development expenses are tracked on a program-by-program basis from the point a program becomes a clinical candidate for us and consist primarily of external costs, such as fees paid to consultants, central laboratories and contractors in connection with our preclinical activities. We do not allocate employee costs, costs associated with our technology or facility expenses, including depreciation or other indirect costs, to specific programs because these costs are currently deployed across multiple product development programs and, as such, are not separately classified. We use internal resources to manage our development activities and our employees work across multiple development programs and, therefore, we do not track their costs by program.

The table below summarizes our research and development expenses incurred by development program:

	Three months ended March 31,		Year ended December 31,	
	2019	2018	2018	2017
			(in thousands)	
STK-001	$ 1,417	$ 92	$1,960	$ —
Non-program specific and unallocated research and development expenses	2,716	1,160	6,411	3,598
Total research and development expenses	$ 4,133	$ 1,252	$8,371	$3,598

We expense all research and development costs in the periods in which they are incurred. Costs for certain development activities are recognized based on an evaluation of the progress to completion of specific tasks using information and data provided to us by our vendors and third-party service providers.

We expect that our expenses will increase substantially in connection with our planned discovery work, preclinical and clinical development activities in the near term and our planned clinical trials in the future. At this time, we cannot reasonably estimate the costs for completing the preclinical and clinical development of any of our other product candidates. We expect our research and development expenses to increase substantially for the foreseeable future as we continue to invest in research and development activities related to developing our product candidates, including investments in manufacturing, as our programs advance into later stages of development and we conduct clinical trials. The process of conducting the necessary clinical research to obtain regulatory approval is costly and time-consuming, and the successful development of our product candidates is highly uncertain. As a result, we are unable to determine the duration and completion costs of our research and development projects or when and to what extent we will generate revenue from the commercialization and sale of any of our product candidates.

Because of the numerous risks and uncertainties associated with product development, we cannot determine with certainty the duration and completion costs of the current or future preclinical studies and clinical trials or if, when, or to what extent we will generate revenues from the commercialization and sale of our product candidates. We may never succeed in achieving regulatory approval for our product candidates. The duration, costs and timing of preclinical studies and clinical trials and development of our product candidates will depend on a variety of factors, including:

- successful completion of preclinical studies and investigational new drug-enabling studies;
- successful enrollment in, and completion of, clinical trials;
- receipt of regulatory approvals from applicable regulatory authorities;
- furthering our commercial manufacturing capabilities and arrangements with third-party manufacturers;
- obtaining and maintaining patent and trade secret protection and non-patent exclusivity;
- launching commercial sales of our product candidates, if and when approved, whether alone or in collaboration with others;
- acceptance of our product candidates, if and when approved, by patients, the medical community and third-party payors;
- effectively competing with other therapies and treatment options;
- a continued acceptable safety profile following approval;
- enforcing and defending intellectual property and proprietary rights and claims; and
- achieving desirable medicinal properties for the intended indications.

A change in the outcome of any of these factors could mean a significant change in the costs and timing associated with the development of our current and future preclinical and clinical product candidates. For example, if the FDA, or another regulatory authority were to require us to conduct clinical trials beyond those that we currently anticipate will be required for the completion of clinical development, or if we experience significant delays in execution of or enrollment in any of our preclinical studies or clinical trials, we could be required to expend significant additional financial resources and time on the completion of preclinical and clinical development. We expect our research and development expenses to increase for the foreseeable future as we continue the development of product candidates.

General and administrative expenses

General and administrative expenses consist primarily of personnel costs, costs related to maintenance and filing of intellectual property, expenses for outside professional services, including legal, human resources, information technology, audit and accounting services, and facilities and other expenses. Personnel costs consist of salaries, benefits and stock-based compensation expense. We expect our general and administrative expenses to increase over the next several years to support our continued research and development activities, manufacturing activities, increased costs of operating as a public company and the potential commercialization of our product candidates. These increases are anticipated to include increased costs related to the hiring of additional personnel, developing commercial infrastructure, fees to outside consultants, lawyers and accountants, and increased costs associated with being a public company such as expenses related to services associated with maintaining compliance with Nasdaq listing rules and SEC requirements, insurance and investor relations costs.

Other income (expense)

Our other income (expense), includes (i) interest income earned on cash reserves in our operating money-market fund investment accounts and (ii) other items of income (expense), net.

Results of operations for the three months ended March 31, 2019 and 2018

The following table sets forth our results of operations:

	Three months ended March 31,	
	2019	2018
	(in thousands)	
Consolidated statements of operations:		
Revenue	$ —	$ —
Operating expenses:		
Research and development	4,133	1,252
General and administrative	2,189	660
Total operating expenses	6,322	1,912
Loss from operations	(6,322)	(1,912)
Other income (expense):		
Interest income	580	—
Other expense, net	—	—
Total other income (expense)	580	—
Net loss	$ (5,742)	$ (1,912)

Research and development expenses

Research and development expenses were $4.1 million for the three months ended March 31, 2019 as compared to $1.3 million for the three months ended March 31, 2018, an increase of $2.8 million. The table below summarizes our research and development expenses:

	Three months ended March 31,	
	2019	**2018**
STK-001	$ 1,417	$ 92
Personnel-related expenses	1,527	706
Third-party services	345	178
Scientific consulting	44	25
Facilities and other research and development expenses	800	251
Total research and development expenses	$ 4,133	$ 1,252

The increase in research and development expenses were primarily attributable to an increase of $1.3 million on our STK-001 program, comprised of third-party services and scientific consulting fees, an increase of $0.8 million in personnel costs resulting from an increase in headcount, an increase of $0.2 million in consulting, third-party services, materials and other costs as we advance our discovery and preclinical activities, and an increase of $0.5 million in facilities and other costs resulting from the growth in our research and development personnel.

General and administrative expenses

General and administrative expenses were $2.2 million for the three months ended March 31, 2019 as compared to $0.7 million for the three months ended March 31, 2018, an increase of $1.5 million.

The increase in general and administrative expenses were primarily attributable to an increase of $0.3 million in personnel costs resulting from an increase in headcount, an increase of $0.6 million in third-party services to support our in-house personnel in various aspects of developing and supporting the business including human resources, information technology, audit, tax, public relations, communications and other general and administrative activities, an increase of $0.3 million related to cost of maintaining and filing of our intellectual property and an increase of $0.3 million in facilities and other costs resulting from the growth in our general and administrative personnel.

Other income (expense)

The change in our other income (expense) for the three months ended March 31, 2019 as compared to the three months ended March 31, 2018 principally reflects returns on higher levels of cash reserves.

Results of operations for the years ended December 31, 2018 and 2017

The following table sets forth our results of operations:

	Year ended December 31,	
	2018	2017
	(in thousands)	
Consolidated statements of operations:		
Revenue	$ —	$ —
Operating expenses:		
Research and development	8,371	3,598
General and administrative	4,410	1,956
Total operating expenses	12,781	5,554
Loss from operations	(12,781)	(5,554)
Other income (expense):		
Interest income	270	—
Other expense, net	(10)	(4)
Total other income (expense)	260	(4)
Net loss	$(12,521)	$(5,558)

Research and development expenses

Research and development expenses were $8.4 million for the year ended December 31, 2018 as compared to $3.6 million for the year ended December 31, 2017, an increase of $4.8 million. The table below summarizes our research and development expenses:

	Year Ended December 31,	
	2018	2017
STK-001	$1,960	$ —
Personnel-related expenses	3,825	1,823
Third-party services	1,680	1,199
Scientific consulting	161	160
Facilities and other research and development expenses	745	416
Total research and development expenses	$8,371	$3,598

The increases in research and development expenses were primarily attributable to an increase of $2.0 million on our STK-001 program, comprised of third-party services and scientific consulting fees, an increase of $2.0 million in personnel costs resulting from an increase in headcount, an increase of $0.5 million in third-party services, materials and other costs as we advance our discovery and preclinical activities, and an increase of $0.3 million in facilities and other costs resulting from the growth in our research and development personnel.

General and administrative expenses

General and administrative expenses were $4.4 million for the year ended December 31, 2018 as compared to $2.0 million for the year ended December 31, 2017, an increase of $2.4 million.

The increases in general and administrative expenses were primarily attributable to an increase of $0.8 million in personnel costs resulting from an increase in headcount, an increase of $0.5 million in third-party services to support our in-house personnel in various aspects of developing and supporting the business including human resources, information technology, audit, tax, public relations, communications and other general and administrative activities, an increase of $0.7 million related to cost of maintaining and filing of our intellectual property and an increase of $0.4 million in facilities and other costs resulting from the growth in our general and administrative personnel.

Other income (expense)

The change in our other income (expense) the year ended December 31, 2018 as compared to the year ended December 31, 2017 principally reflects returns on higher levels of cash reserves.

Liquidity and capital resources

Since our inception through March 31, 2019, our operations have been financed by net proceeds of $131.0 million primarily from the sale of convertible notes and our convertible preferred stock. As of March 31, 2019, we had $98.9 million in cash, cash equivalents and restricted cash. Cash in excess of immediate requirements is invested in accordance with our investment policy, primarily with a view to liquidity and capital preservation.

We have incurred losses since our inception in June 2014 and, as of March 31, 2019, we had an accumulated deficit of $31.5 million. Our primary use of cash is to fund operating expenses, which consist primarily of research and development expenditures, and to a lesser extent, general and administrative expenditures. Cash used to fund operating expenses is impacted by the timing of when we pay these expenses, as reflected in the change in our outstanding accounts payable and accrued expenses.

Our product candidates may never achieve commercialization and we anticipate that we will continue to incur losses for the foreseeable future. We expect that our research and development expenses, general and administrative expenses, and capital expenditures will continue to increase. As a result, until such time, if ever, as we can generate substantial product revenue, we expect to finance our cash needs through a combination of equity offerings, debt financings or other capital sources, including potentially collaborations, licenses and other similar arrangements. Our primary uses of capital are, and we expect will continue to be, compensation and related expenses, third-party clinical research and development services, costs relating to the build-out of our headquarters and manufacturing facility, license payments or milestone obligations that may arise, laboratory and related supplies, clinical costs, manufacturing costs, legal and other regulatory expenses and general overhead costs.

Based upon our current operating plan, we believe that the net proceeds from this offering, together with our existing cash, cash equivalents and restricted cash as of March 31, 2019 will enable us to fund our operating expenses and capital expenditure requirements through at least the end of 2022. We have based this estimate on assumptions that may prove to be wrong, and we could utilize our available capital resources sooner than we currently expect. We will continue to require additional financing to advance our current product candidates through clinical development, to develop, acquire or in-license other potential product candidates and to fund operations for the foreseeable future. We will continue to seek funds through equity offerings, debt financings or other capital sources, including potentially collaborations, licenses and other similar arrangements. However, we may be unable to raise additional funds or enter into such other arrangements when needed on favorable terms or at all. If we do raise additional capital through public or private equity offerings, the ownership interest of our existing stockholders, including investors in this offering, will be diluted, and the terms of these securities may include liquidation or other preferences that adversely affect our stockholders' rights. If we raise additional capital through debt financing, we may be subject to covenants limiting or

restricting our ability to take specific actions, such as incurring additional debt, making capital expenditures or declaring dividends. Any failure to raise capital as and when needed could have a negative impact on our financial condition and on our ability to pursue our business plans and strategies. If we are unable to raise capital, we will need to delay, reduce or terminate planned activities to reduce costs.

Because of the numerous risks and uncertainties associated with research, development and commercialization of pharmaceutical products, we are unable to estimate the exact amount of our operating capital requirements. Our future funding requirements will depend on many factors, including, but not limited to:

- the scope, progress, results and costs of researching and developing our lead product candidates or any future product candidates, and conducting nonclinical studies and clinical trials;
- the timing of, and the costs involved in, obtaining regulatory approvals or clearances for our lead product candidates or any future product candidates;
- the number and characteristics of any additional product candidates we develop or acquire;
- the timing of any cash milestone payments if we successfully achieve certain predetermined milestones;
- the cost of manufacturing our lead product candidates or any future product candidates and any products we successfully commercialize, including costs associated with building-out our manufacturing capabilities;
- our ability to establish and maintain strategic collaborations, licensing or other arrangements and the financial terms of any such agreements that we may enter into;
- the expenses needed to attract and retain skilled personnel;
- the costs associated with being a public company; and
- the timing, receipt and amount of sales of any future approved or cleared products, if any.

Further, our operating plans may change, and we may need additional funds to meet operational needs and capital requirements for clinical trials and other research and development activities. We currently have no credit facility or committed sources of capital. Because of the numerous risks and uncertainties associated with the development and commercialization of our product candidates, we are unable to estimate the amounts of increased capital outlays and operating expenditures associated with our current and anticipated product development programs.

Cash flows

The following table summarizes our cash flows:

	Three months ended March 31,		Year ended December 31,	
	2019	2018	2018	2017
				(in thousands)
Net cash (used in) provided by:				
Operating activities	$ (6,532)	$ (1,989)	$ (10,964)	$(5,384)
Investing activities	(90)	(26)	(925)	(113)
Financing activities	(68)	12,419	115,639	5,974
Net increase (decrease) in cash, cash equivalents and restricted cash	$ (6,690)	$ 10,404	$103,750	$ 477

Operating activities

During the three months ended March 31, 2019, cash used in operating activities was $6.5 million and was primarily attributable to a net loss of $5.7 million, partially offset by non-cash charges of $0.3 million for share-based compensation and depreciation, and a net change of $1.1 million in our net operating assets and liabilities.

During the three months ended March 31, 2018, cash used in operating activities was $2.0 million and was attributable to a net loss of $1.9 million and a net change of $0.1 million in our net operating assets and liabilities.

During the year ended December 31, 2018, cash used in operating activities was $11.0 million and was attributable to a net loss of $12.5 million, partially offset by non-cash charges of $0.5 million for share-based compensation and depreciation, and a net change of $1.0 million in our net operating assets and liabilities.

During the year ended December 31, 2017, cash used in operating activities was $5.4 million and was attributable to a net loss of $5.6 million, partially offset by non-cash charges of $0.1 million and a net change of $0.1 million in our net operating assets and liabilities.

Investing activities

Our investing activities during the years ended December 31, 2018 and 2017 and during the three months ended March 31, 2019 and 2018 have consisted principally of purchases of property and equipment.

Financing activities

Our financing activities during the three months ended March 31, 2019 consisted primarily of deferred offering costs related to the IPO offset by proceeds from the issuance of common stock.

Our financing activities during the three months ended March 31, 2018 consisted primarily of the closing of our Series A-2 convertible preferred stock financing in January 2018.

Our financing activities during the year ended December 31, 2018 included closings on our Series A-2 convertible preferred stock financing in January and September 2018 aggregating gross proceeds of $26.0 million, and the sale of Series B convertible preferred stock in October 2018 raising gross proceeds of $90.0 million.

Our financing activities during the year ended December 31, 2017 included a second extension on our Series A convertible preferred stock financing in February 2017 with gross proceeds of $3.0 million and proceeds on a $3.0 million simple agreement for future equity, or SAFE, in October 2017 which was converted in the initial closing of our Series A-2 convertible preferred stock financing in January 2018.

Contractual obligations and commitments

The following table summarizes our contractual obligations as of December 31, 2018 and the effects that such obligations are expected to have on our liquidity and cash flows in future periods:

		Payments Due by Period			
	Total	Less Than 1 Year	1 to 3 Years	4 to 5 Years	More than 5 Years
					(in thousands)
Operating lease obligations	$3,373	$ 1,051	$2,251	$ 71	$ —
Total	$3,373	$ 1,051	$2,251	$ 71	$ —

In August 2018, we entered into an agreement to sublease approximately 23,000 square feet of space for a term of three years. Lease terms are triple net lease commencing at $0.9 million per year, then with 3% annual base rent increases plus operating expenses, real estate taxes, utilities and janitorial fees. The lease commencement date was December 10, 2018.

In December 2018, we entered into an agreement to lease 2,485 square feet of space for a term of three years. The lease includes one renewal option for an additional two years. Lease terms commence at $0.2 million per year, with 2.5% annual base rent increases plus operating expenses, real estate taxes, utilities and janitorial fees. We occupied this space in May 2019.

Commitments

Our commitments primarily consist of obligations under our agreements with CSHL and the University of Southampton. As of March 31, 2019, we were unable to estimate the timing or likelihood of achieving the milestones or making future product sales. For additional information regarding our agreements, see “Business—License agreements.”

Additionally, we have entered into agreements with third-party contract manufacturers for the manufacture and processing of certain of our product candidates for preclinical testing purposes, and we have entered and will enter into other contracts in the normal course of business with contract research organizations for clinical trials and other vendors for other services and products for operating purposes. These agreements generally provide for termination or cancellation, other than for costs already incurred.

Off-balance sheet arrangements

During the periods presented, we did not have, nor do we currently have, any off-balance sheet arrangements as defined under SEC rules.

Critical accounting policies and significant judgments and estimates

Our management’s discussion and analysis of our financial condition and results of operations is based on our consolidated financial statements, which have been prepared in accordance with generally accepted accounting principles, or GAAP. The preparation of these financial statements requires us to make estimates and assumptions that affect the reported amounts of assets and liabilities and the disclosure of contingent assets and liabilities at the date of the consolidated financial statements, as well as the reported expenses incurred during the reporting periods. Our estimates are based on our historical experience and on various other factors that we believe are reasonable under the circumstances, the results of which form the basis for making judgments about the carrying value of assets and liabilities that are not readily apparent from other sources. Actual results may differ from these estimates under different assumptions or conditions. We believe that the accounting policies discussed below are critical to understanding our historical and future performance, as these policies relate to the more significant areas involving management’s judgments and estimates.

Stock-based compensation

We recognize compensation costs related to share-based awards granted to employees and directors, including stock options and vesting restricted stock, based on the estimated fair value of the awards on the date of grant. We estimate the grant date fair value, and the resulting stock-based compensation, using the Black-Scholes option-pricing model. The grant date fair value of the stock-based awards is recognized on a straight-line basis over the requisite service period, which is generally the vesting period of the respective awards.

The Black-Scholes option-pricing model requires the use of subjective assumptions to determine the fair value of stock-based awards. These assumptions include:

- *Fair value of common stock*—Historically, for all periods prior to this initial public offering, the fair value of the shares of common stock underlying our share-based awards was estimated on each grant date by our board of directors. To determine the fair value of our common stock underlying option grants, our board of directors considered, among other things, valuations of our common stock prepared by an unrelated third-party valuation firm in accordance with the guidance provided by the American Institute of Certified Public Accountants Practice Guide, *Valuation of Privately-Held-Company Equity Securities Issued as Compensation*, or the *Practice Aid*.
- *Expected term*—The expected term represents the period that stock-based awards are expected to be outstanding. The expected term for option grants is determined using the simplified method. The simplified method deems the expected term to be the midpoint between the vesting date and the contractual life of the stock-based awards.
- *Expected volatility*—Since we have been a privately held company and do not have any trading history for our common stock, the expected volatility is estimated based on the average volatility for comparable publicly traded biotechnology companies over a period equal to the expected term of the stock option grants. The comparable companies were chosen based on their similar size, stage in the life cycle or area of specialty. We will continue to apply this process until a sufficient amount of historical information regarding the volatility of our own stock price becomes available.
- *Risk-free interest rate*—The risk-free interest rate is based on the U.S. Treasury zero coupon issues in effect at the time of grant for periods corresponding with the expected term of option.
- *Expected dividend*—We have never paid dividends on our common stock and have no plans to pay dividends on our common stock. Therefore, we used an expected dividend yield of zero.

The following table presents the weighted-average assumptions used to estimate the fair value of share-based awards granted:

	Three months ended March 31,		Year ended December 31,	
	2019	2018	2018	2017
Risk-free interest rate	2.32-2.81%	—	2.67-2.84%	2.27%
Expected dividend yield	0%	—	0%	0%
Expected life	6.375 years	—	6.25-6.375 years	6.25-6.375 years
Expected volatility	57-61%	—	57-60%	65%

We will continue to use judgment in evaluating the assumptions utilized for our share-based compensation expense calculations on a prospective basis. In addition to the assumptions used in the Black-Scholes option-pricing model, the amount of stock-based compensation expense we recognize in our consolidated financial statements includes actual stock option forfeitures.

Determination of the fair value of common stock

Historically, for all periods prior to this offering, the fair values of the shares of common stock underlying our share-based awards were estimated on each grant date by our board of directors. In order to determine the fair value of our common stock our board of directors considered, among other things, contemporaneous valuations of our common stock prepared by an independent third-party valuation specialist in accordance with the guidance provide by the Practice Aid.

Given the absence of a public trading market for our common stock, our board of directors exercised their judgment and considered a number of objective and subjective factors to determine the best estimate of the fair value of our common stock, including contemporaneous valuations performed by an independent third party, our stage of development, important developments in our operations, the prices at which we sold shares of our preferred stock, the rights, preferences and privileges of our preferred stock relative to those of our common stock, actual operating results and financial performance, the conditions in the biotechnology industry and the economy in general, the stock price performance and volatility of comparable public companies, and the lack of liquidity of our common stock, among other factors. After the closing of this offering, our board of directors will determine the fair value of each share of underlying common stock based on the closing price of our common stock as reported on the date of the grant. Our board of directors intended all options granted to be exercisable at a price per share not less than the per share fair value of our common stock underlying those options on the grant date.

We performed common stock valuations, with the assistance of an independent third-party valuation specialist, as of August 2016, January 2018, October 2018 and February 2019 which resulted in a valuation of our common stock of $0.40, $0.60, $2.19 and $4.48, respectively. In conducting the valuations, the independent third-party valuation specialist considered all objective and subjective factors that it believed to be relevant for each valuation conducted in accordance with the Practice Aid, including our best estimate of our business condition, prospects and operating performance at each valuation date. Other significant factors included:

- the prices of our preferred stock sold to outside investors in arm's length transactions, and the rights, preferences and privileges of our preferred stock as compared to those of our common stock, including the liquidation preferences of our preferred stock;
- our stage of development and business strategy and the material risks related to our business and industry;
- the valuation of publicly traded companies in the life sciences and biotechnology sectors, as well as recently completed mergers and acquisitions of guideline companies;
- our results of operations and financial position;
- the composition of, and changes to, our management team and board of directors;
- the lack of liquidity of our common stock;
- any external market conditions affecting the life sciences and biotechnology industry sectors;
- the likelihood of achieving a liquidity event for the holders of our common stock and stock options, such as an initial public offering, or IPO, or a sale of our company, given prevailing market conditions; and
- the state of the IPO market for similarly situated privately held life sciences companies.

For the August 2016 valuation, we employed an option pricing method, or OPM, framework and utilized a guideline transactions market approach for inferring the equity value implied by a selection of guideline transactions. This method was selected as there was no recent arm's-length financing transaction and, as of the valuation date, we were at an early stage of development and future liquidity events were difficult to forecast. Application of OPM involves making assumptions for the expected time to liquidity, volatility and risk-free rate and then solving for the value of equity such that value for the most recent financing equals the amount paid. For the August 2016 valuation we assumed a weighted average cost of capital of 40% and a 3.25-year term to a liquidity event to estimate total equity value and, for purposes of the OPM allocation of total equity value, a 65% volatility rate and a 1.38-year estimated term. We then reflected a probability weighted average discount for lack of marketability of 35% to arrive at a $0.40 per share valuation of our common stock.

For the January 2018 valuation, we employed an OPM framework and utilized the back-solve method for inferring and allocating the equity value predicated on the capital raise that transpired just prior to the valuation date. This method was selected as management concluded that the recent financing transaction was an arm's-length transaction. Furthermore, as of the valuation date we were at an early stage of development and future liquidity events were difficult to forecast. Application of the OPM back-solve method involves making assumptions for the expected time to liquidity, volatility and risk-free rate and then solving for the value of equity such that value for the most recent financing equals the amount paid. For the January 2018 valuation and for purposes of the OPM allocation of total equity value determined with reference to a recent financing transaction, we assumed a 57% volatility rate and a 1.5-year estimated term. We then reflected a probability weighted average discount for lack of marketability of 35% to arrive at a $0.60 per share valuation of our common stock.

For the October 2018 valuation, the independent third-party valuation specialist used a hybrid method of two potential liquidity outcomes: a trade-sale scenario predicated on the arm's length capital raise that transpired just prior to the valuation date and an IPO scenario with reference to recent IPO transactions in the biotechnology and pharmaceutical industry and considering our preclinical stage of development. Under the hybrid method, the per share value calculated under the two scenarios are weighted based on expected exit outcomes and the quality of the information specific to each allocation methodology to arrive at a final estimated fair value per share value of the common stock before a discount for lack of marketability is applied. For the October 2018 valuation we (i) assigned a 90% probability of occurrence to the trade-sale scenario, with a 60% volatility rate and a 2-year estimated term applied within the OPM, then reflected a probability weighted average discount for lack of marketability of 35%; and (ii) we assigned a 10% probability of occurrence to the IPO scenario, with a 30% weighted average cost of capital and a 0.88-year estimated term to an IPO event, then reflected a probability weighted average discount for lack of marketability of 15%.

For the February 2019 valuation, the independent third-party valuation specialist used a hybrid method of two potential liquidity outcomes: a trade-sale scenario predicated on the arm's length capital raise that transpired prior to the valuation date and an IPO scenario with reference to recent IPO transactions in the biotechnology and pharmaceutical industry and considering our preclinical stage of development. Under the hybrid method, the per share values calculated under the two scenarios are weighted based on expected exit outcomes and the quality of the information specific to each allocation methodology to arrive at a final estimated fair value per share value of the common stock before a discount for lack of marketability is applied. For the February 2019 valuation, we (i) assigned a 65% probability of occurrence to the trade-sale scenario, with a 61% volatility rate and a 1.75-year estimated term applied within the OPM, then reflected a probability weighted average discount for lack of marketability of 30%; and (ii) we assigned a 35% probability of occurrence to the IPO scenario, with a 25% weighted average cost of capital and a 0.38-year estimated term to an IPO event, then reflected a probability weighted average discount for lack of marketability of 12.5%.

For the April 2019 valuation, the independent third-party valuation specialist again used a hybrid method of two potential liquidity outcomes: a trade-sale scenario predicated on the Series B Financing and an IPO scenario with reference to recent IPO transactions in the biotechnology and pharmaceutical industry and considering the Company's preclinical stage of development. Under the hybrid method, the per share values calculated under the two scenarios were weighted based on expected exit outcomes and the quality of the information specific to each allocation methodology to arrive at a final estimated fair value per share value of the Company's common stock before a DLOM is applied. For the April 2019 valuation, the Company (i) assigned a 40% probability of occurrence to the trade-sale scenario, with a 61% volatility rate and a 1.75-year estimated term applied within the OPM, then reflected a probability weighted average DLOM of 25%; and (ii) the Company assigned a 60% probability of occurrence to the IPO scenario, with a 25% weighted average cost of capital and a 0.25-year estimated term to an IPO event, then reflected a probability weighted average DLOM of 7.5%.

The estimates of fair value of our common stock are highly complex and subjective. There are significant judgments and estimates inherent in the determination of the fair value of our common stock. These judgments and estimates include assumptions regarding our future operating performance, the time to completing an IPO or other liquidity event, the related valuations associated with these events, and the determinations of the appropriate valuation methods at each valuation date. The assumptions underlying these valuations represent management's best estimates, which involve inherent uncertainties and the application of management judgment. If we had made different assumptions, our stock-based compensation expense, net loss and net loss per share applicable to common stockholders could have been materially different.

For valuations after the completion of this offering, our board of directors will determine the fair value of each share of underlying common stock based on the closing price of our common stock as reported on the date of grant.

Determination of estimated offering price

We and our underwriters determined the estimated price range set forth on the cover of this preliminary prospectus, which is $14.00 to $16.00 per share. In comparison, our estimate of the fair value of our common stock was $6.83 per share at April 16, 2019, which was determined by our board of directors with the assistance of an independent third-party valuation of our common shares.

We note that, as is typical in initial public offerings, the estimated price range for this offering was not derived using a formal determination of fair value but was determined based upon discussions between us and the underwriters. Among the factors considered in setting the estimated range were prevailing market conditions, estimates of our business potential, progress in our clinical trials and developments in our business, the general condition of the securities market and the market prices of, and demand for, publicly-traded common stock of generally comparable companies.

The intrinsic value of all outstanding options as of March 31, 2019 was $53.9 million based on an assumed initial public offering price of $15.00 per share, the midpoint of the price range set forth on the cover of this prospectus.

Emerging growth company and smaller reporting company status

We are an "emerging growth company," as defined in the Jumpstart our Business Startups Act of 2012, or the JOBS Act. Under the JOBS Act, emerging growth companies can delay adopting new or revised accounting standards issued subsequent to the enactment of the JOBS Act until such time as those standards apply to private companies.

We have elected to use this extended transition period for complying with new or revised accounting standards that have different effective dates for public and private companies until the earlier of the date we (i) are no longer an emerging growth company or (ii) affirmatively and irrevocably opt out of the extended transition period provided in the JOBS Act.

As a result, our consolidated financial statements may not be comparable to companies that comply with new or revised accounting pronouncements as of public company effective dates.

We will remain an emerging growth company until the earliest of (i) the last day of our first fiscal year (a) following the fifth anniversary of the completion of this offering, (b) in which we have total annual gross revenues of at least $1.07 billion, or (c) when we are deemed to be a large accelerated filer, which means the market value of our common stock that is held by non-affiliates exceeds $700 million as of the prior June 30th and (ii) the date on which we have issued more than $1.0 billion in non-convertible debt securities during the prior three-year period.

We are also a “smaller reporting company,” meaning that the market value of our stock held by non-affiliates plus the proposed aggregate amount of gross proceeds to us as a result of this offering is less than $700 million and our annual revenue is less than $100 million during the most recently completed fiscal year. We may continue to be a smaller reporting company after this offering if either (i) the market value of our stock held by non-affiliates is less than $250 million or (ii) our annual revenue is less than $100 million during the most recently completed fiscal year and the market value of our stock held by non-affiliates is less than $700 million. If we are a smaller reporting company at the time we cease to be an emerging growth company, we may continue to rely on exemptions from certain disclosure requirements that are available to smaller reporting companies. Specifically, as a smaller reporting company we may choose to present only the two most recent fiscal years of audited financial statements in our Annual Report on Form 10-K and, similar to emerging growth companies, smaller reporting companies have reduced disclosure obligations regarding executive compensation.

Recently adopted accounting pronouncements

In May 2017, the FASB issued ASU 2017-09, *Compensation—Stock Compensation (Topic 718): Scope of Modification Accounting*, which provides guidance about which changes to the terms or conditions of a share-based payment award require an entity to apply modification accounting in Topic 718. The amendments in this ASU should be applied prospectively to an award modified on or after the adoption date. We adopted ASU 2017-09 effective January 1, 2018, and the adoption of ASU 2017-09 did not impact our consolidated financial statements or financial statement disclosures.

Recently issued accounting pronouncements

In February 2016, the FASB issued ASU 2016-02, *Leases* (Topic 842), with guidance regarding the accounting for and disclosure of leases. The update requires lessees to recognize all leases, including operating leases, with a term greater than 12 months on the balance sheet. This update also requires lessees and lessors to disclose key information about their leasing transactions. This guidance will be effective for public companies for annual and interim periods beginning after December 15, 2018. For all other companies, this standard is effective for annual reporting periods beginning after December 15, 2019, and interim periods within annual periods beginning after December 15, 2020. We will adopt this standard on January 1, 2020. While we expect the implementation of ASU 2016-02 to result in the recognition of right-of-use assets and lease liabilities for leased facilities, we are still evaluating the impact that the adoption of ASU 2016-02 will have on our consolidated financial statements.

In July 2017, the FASB issued ASU 2017-11, *Earnings Per Share* (Topic 260), Distinguishing Liabilities from Equity (Topic 480) and Derivatives and Hedging (Topic 815): I. *Accounting for Certain Financial Instruments with Down Round Features; II. Replacement of the Indefinite Deferral for Mandatorily Redeemable Financial Instruments of Certain Nonpublic Entities and Certain Mandatorily Redeemable Noncontrolling Interests with a Scope Exception.* Part I of this update addresses the complexity of accounting for certain financial instruments with down round features. Down round features are features of certain equity-linked instruments (or embedded features) that result in the strike price being reduced on the basis of the pricing of future equity offerings. Current accounting guidance creates cost and complexity for entities that issue financial instruments (such as warrants and convertible instruments) with down round features that require fair value measurement of the entire instrument or conversion option. Part II of this update addresses the difficulty of navigating Topic 480, Distinguishing Liabilities from Equity, because of the existence of extensive pending content in the FASB Accounting Standards Codification. This pending content is the result of the indefinite deferral of accounting requirements about mandatorily redeemable financial instruments of certain nonpublic entities and certain mandatorily redeemable noncontrolling interests. The amendments in Part II of this update do not have an accounting effect. For public business entities, the amendments in Part I of ASU 2017-11 are effective for fiscal

years and interim periods within those years beginning after December 15, 2018. For all other entities, the amendments in Part I of this update are effective for fiscal years beginning after December 15, 2019, and interim periods within fiscal years beginning after December 15, 2020. We intend to adopt Part I of this update on January 1, 2020. Early adoption is permitted for all entities, including adoption in an interim period. We are currently assessing the potential impact of adopting ASU 2017-11 on our consolidated financial statements and financial statement disclosures.

In August 2018, the FASB issued ASU 2018-13, *"Fair Value Measurement (Topic 820), Disclosure Framework—Changes to the Disclosure Requirements for Fair Value Measurement".* This ASU removed the following disclosure requirements: (i) the amount of and reasons for transfers between Level 1 and Level 2 of the fair value hierarchy; (ii) the policy for timing of transfers between levels; and (i) the valuation processes for Level 3 fair value measurements. Additionally, this update added the following disclosure requirements: (i) the changes in unrealized gains and losses for the period included in other comprehensive income and loss for recurring Level 3 fair value measurements held at the end of the reporting period; and (ii) the range and weighted average of significant unobservable inputs used to develop Level 3 fair value measurements. For certain unobservable inputs, an entity may disclose other quantitative information (such as the median or arithmetic average) in lieu of the weighted average if the entity determines that other quantitative information would be a more reasonable and rational method to reflect the distribution of unobservable inputs used to develop Level 3 fair value measurements. ASU 2018-13 will be effective for all entities, for fiscal years beginning after December 15, 2019 with early adoption permitted. We intend to adopt this standard on January 1, 2020 and does not expect that the adoption of the update will have a material impact on our consolidated financial statements.

Quantitative and qualitative disclosures about market risk

Interest rate risk

We are exposed to market risks in the ordinary course of our business. These risks primarily include interest rate sensitivities. We held cash, cash equivalents and restricted cash of $105.6 million as of December 31, 2018 and $98.9 million as of March 31, 2019. We generally hold our cash in interest-bearing money market accounts. Our primary exposure to market risk is interest rate sensitivity, which is affected by changes in the general level of U.S. interest rates. An immediate 100 basis point change in interest rates would affect the fair market value of our cash equivalents by approximately $1.1 million.

Business

Overview

We are pioneering a new way to treat the underlying causes of severe genetic diseases by precisely upregulating protein expression. We are developing novel antisense oligonucleotide, or ASO, medicines that target ribonucleic acid, or RNA, and modulate precursor-messenger RNA, or pre-mRNA, splicing to upregulate protein expression where needed and with appropriate specificity to near normal levels. We utilize our proprietary technology platform, Targeted Augmentation of Nuclear Gene Output, or TANGO, to design ASOs to upregulate the expression of protein by individual genes in a patient. Our approach is designed to allow us to deliver in a highly precise, durable and controlled manner disease-modifying therapies to a wide range of relevant tissues, including the central nervous system, or CNS, eye, kidney and liver. We designed our lead product candidate, STK-001, to treat Dravet syndrome, a severe and progressive genetic epilepsy. With a well-defined patient population based on routine genetic testing and learnings from recently approved drugs for the treatment of Dravet syndrome to inform the clinical and regulatory pathways for STK-001, we anticipate an efficient clinical program for STK-001. We plan to submit an investigational new drug application, or IND, for STK-001 by early 2020 and expect to initiate a Phase 1/2 clinical trial in the first half of 2020. We intend to nominate a second candidate to treat an additional genetic disease for preclinical development by the first half of 2020.

Our proprietary technology platform is based on the pioneering work conducted on pre-mRNA splicing and ASOs in the laboratory of one of our co-founders, Adrian R. Krainer, Ph.D., of Cold Spring Harbor Laboratory in New York. Inspired by the clinical success of SPINRAZA, an ASO medicine for the treatment of spinal muscular atrophy that was co-invented by Professor Krainer, our company was founded to develop a general antisense approach to upregulate protein expression. TANGO exploits unique, patented mechanisms for antisense-mediated modulation of splicing to prevent the synthesis of naturally occurring non-productive messenger RNA, or mRNA, and to increase the synthesis of productive mRNA to increase production of functional protein. Our technology is amenable to a large number of mutations and can thereby potentially provide a single-drug approach for diseases that are caused by many loss-of-function mutations in a single gene. We have identified approximately 2,900 monogenic, or single gene, diseases which we believe are amenable to TANGO. We have an intellectual property estate that includes multi-national allowed and pending claims for the TANGO mechanisms, as well as multi-national pending claims relating to compositions of matter of oligonucleotides designed to target specific TANGO elements in genes for more than 140 genetic diseases that we believe are amenable to upregulation of target protein expression using TANGO.

We are initially focused on applying the transformative potential of our platform to develop precision medicines for autosomal dominant haploinsufficiency diseases. There are more than 660 known monogenic diseases that are categorized as haploinsufficiencies. These diseases are ones in which only one copy, or allele, of the gene needs to be mutated for the disease or trait to develop, and that mutated allele generates a protein that is severely deficient in amount or activity, resulting in approximately 50% of normal protein expression in the patient. We believe TANGO is well-suited to treat haploinsufficiencies by increasing expression of the healthy, or wild-type, allele, thereby restoring the target protein to near normal levels.

We are developing TANGO as potentially the first precision medicine platform for a category of severe genetic diseases known as autosomal dominant haploinsufficiencies. Existing precision medicine platforms, including gene therapy, gene editing, modified mRNA, protein-based drugs, small molecules and oligonucleotides, have fundamental limitations that make them poorly suited to address haploinsufficiencies. Numerous technical challenges preclude effective application of these modalities to haploinsufficiencies, including: (i) the inability to control level and tissue distribution of target protein expression, (ii) potential irreversible on- and off-target effects, (iii) target gene size limitations, (iv) incompatibility with diseases caused by many mutations, (v) drug

manufacturing and (vi) delivery hurdles. There is a need for novel therapeutics that can restore protein expression and address the underlying genetic causes of haploinsufficiencies.

Within haploinsufficiency diseases, we are initially prioritizing the development of ASOs for the treatment of genetic epilepsies. According to a 2010 publication in *Nature Reviews Neurology* and a 2018 publication in *JAMA Neurology*, more than 50% of epilepsies are now recognized as having a genetic basis and more than 30% of patients are refractory to existing therapies, especially those with a genetic epilepsy. Our most advanced program is the potentially first disease-modifying therapy for Dravet syndrome, a severe and progressive genetic epilepsy. Dravet syndrome is caused by loss-of-function mutations in one allele of the *SCN1A* gene and is characterized by frequent and prolonged seizures beginning in the first year of life, severe intellectual and developmental disabilities and other serious health problems, including, notably, sudden premature death in approximately 20% of patients with Dravet syndrome. Current treatments for Dravet syndrome only address the occurrence of seizures, not the underlying cause of disease. According to a 2017 study as published in the *Developmental Medicine & Child Neurology Journal*, more than 90% of Dravet syndrome patients still report suffering from incomplete seizure control with existing antiepileptic regimens.

We have generated preclinical data demonstrating proof-of-mechanism for STK-001 and intend to submit an IND application by early 2020. With a well-defined patient population based on routine genetic testing and learnings from recently approved drugs for the treatment of Dravet syndrome to inform the clinical and regulatory pathways for STK-001, we anticipate an efficient clinical program for STK-001. We are leveraging similar ASO chemistry as the approved drug SPINRAZA, which minimizes potential safety and biodistribution risks in the CNS. We expect to initiate a Phase 1/2 clinical trial in children and adolescents with Dravet syndrome in the first half of 2020 and anticipate clinical data, including preliminary efficacy data, in 2021. Beyond STK-001, we are building a pipeline of novel precision medicines for genetic epilepsies and other haploinsufficiencies, and we intend to nominate our second candidate to treat an additional genetic disease for preclinical development by the first half of 2020.

Our executive management team has extensive collective expertise in human genetics and modulation of RNA processes using ASOs, as well as a track record of success in rare disease drug development. Our executive team and co-founders have been previously involved with other companies in the discovery, development and commercialization of many treatments for rare diseases, including Sarepta's Exondys 51 and Biogen's SPINRAZA. Our scientific and clinical advisory boards are comprised of leading experts in the fields of human genetics, pre-mRNA splicing and ASOs, and neurodevelopmental and neurodegenerative diseases. Their involvement in both academic research and clinical practice allows us to gain proprietary and early insight into emerging biology and clinical practice that informs our business strategy. As of March 31, 2019, we have raised over $130 million in funding from two financing rounds, including investments from Apple Tree Partners, RTW Investments, RA Capital Management, Cormorant Asset Management, Perceptive Advisors and funds managed by Janus Henderson Investors, Redmile Group, Sphera Funds Management and Alexandria Venture Investments.

Our strategy

We are using our proprietary TANGO technology platform to create ASOs for the treatment of severe genetic diseases. The critical components of our strategy include:

- *Rapidly advance our lead program, STK-001, to clinical proof-of-concept, approval and commercialization.* We intend to advance our lead product candidate, STK-001, into a Phase 1/2 clinical trial in children and adolescents with Dravet syndrome in the first half of 2020. We are leveraging previously-validated ASO chemistry, a modality that has been successfully utilized for other diseases, a well-defined patient population based on routine genetic testing and learnings from recently approved drugs for the

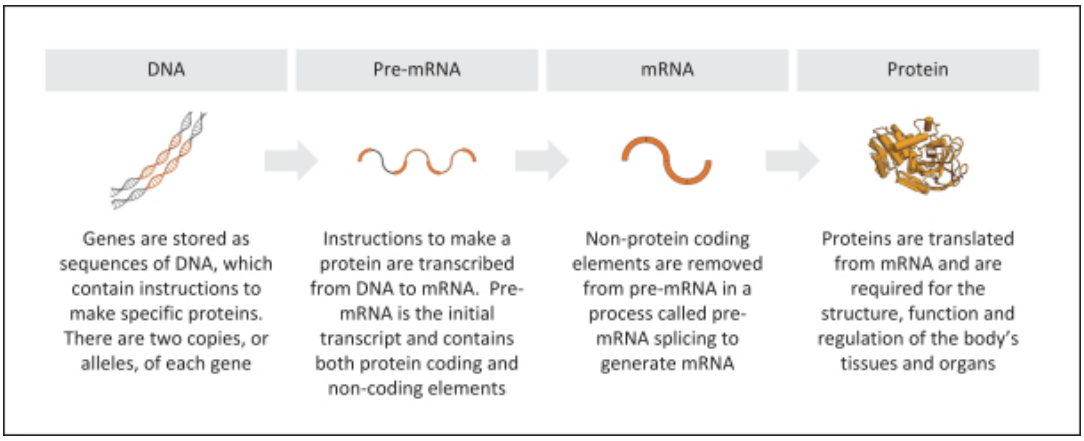
treatment of Dravet syndrome to inform the clinical and regulatory pathways for STK-001 and minimize potential safety concerns and development risk. We believe STK-001 has the potential to significantly reduce both the occurrence of seizures and significant non-seizure comorbidities. If we see evidence of efficacy following clinical data, then we would plan to meet with regulatory authorities to discuss expedited regulatory pathways. If approved, we intend to leverage a lean, targeted internal commercial organization to bring STK-001 to patients globally.

- *Prioritize genetic epilepsies for near-term development efforts.* We believe that no other haploinsufficiency disease area holds as much clear need or as much promise for near-term medical breakthrough as genetic epilepsies. Leveraging our proprietary database, we have identified over 100 genes that are commonly mutated amongst epilepsy patients and that may be amenable to TANGO. We believe that the learnings from our lead Dravet syndrome program will significantly reduce the developmental risk of subsequent programs in our pipeline, particularly those targeting the CNS.
- *Expand our pipeline into other disease areas to fully exploit the potential of our proprietary platform.* We have built a target discovery process utilizing proprietary bioinformatics algorithms and extensive in-house expertise in whole transcriptome RNA sequencing to rapidly and systematically identify diseases that we believe can be addressed using our platform. We are advancing several early programs focused on multiple targets, including haploinsufficiency diseases of the CNS, eye, kidney and liver. Indications beyond genetic epilepsies for which our technology may be applicable include autosomal dominant optic atrophy and autosomal dominant polycystic kidney disease. Longer-term, we believe that our ASOs may have the potential to upregulate non-mutated genes in biological pathways to treat diseases or conditions that are caused by multiple genes or are multifactorial, such as autoimmune diseases, aging and cancer.
- *Maintain broad commercial rights to our product candidates.* We own commercial rights to our technologies and clinical programs, including our lead product candidate, STK-001. We intend to build a fully integrated global biotechnology company and independently pursue the development and commercialization of our key product candidates. As we continue to advance our programs, we may pursue strategic collaborations to share risk and upside in disease areas with higher inherent biology risk, larger clinical trial sizes and longer or more complex clinical and regulatory paths. We plan to opportunistically evaluate potential collaboration arrangements and may elect to enter into an arrangement with a pharmaceutical or biotechnology company as early as this year.
- *Continue to strengthen and expand our intellectual property portfolio.* We have an intellectual property estate that includes multi-national allowed and pending claims for the TANGO mechanisms, as well as multi-national pending claims relating to compositions of matter of oligonucleotides designed to target specific TANGO elements in genes for more than 140 genetic diseases that we believe are amenable to upregulation of target protein expression using TANGO. Our proprietary position is reinforced by additional technical know-how and trade secrets. We continually assess and refine our intellectual property strategy as we identify new targets amenable to TANGO, and we will file additional patent applications as appropriate.

Genetic diseases and precision medicines

Each person's genetic material, or genome, consists of deoxyribonucleic acid, or DNA, in sequences of genetic code called genes. There are two copies, or alleles, of each gene, which act as instructions to produce specific proteins. When a cell needs to produce a protein, the instructions to make that protein are transcribed from DNA to mRNA for each allele. The initial transcript is called pre-mRNA and contains both protein coding and non-coding elements. During transcription, the non-protein coding elements, such as introns or non-coding exons, are removed in a process called pre-mRNA splicing. Splicing serves to stitch together the coding

elements, or exons, to generate mRNA. The mRNA then serves as the instructions to produce protein. Proteins are required for the structure, function, and regulation of the body's tissues and organs. The key steps for protein synthesis are exemplified in the schematic below.



The DNA in the human genome contains approximately three billion nucleotide base pairs, and small changes, or mutations, routinely occur in the base pairs. A mutation in the gene can alter the amount or activity of the protein. Currently, there are estimated to be over 10,000 diseases caused by a genetic abnormality in a single gene. These are also known as monogenic diseases. Monogenic diseases can be categorized as either autosomal recessive or autosomal dominant diseases.

For diseases that are autosomal recessive, both alleles of a gene must be mutated for the disease or trait to develop. The protein that is generated is severely deficient in amount or activity and typically results in less than 10-25% of normal protein expression in the patient. Conversely, autosomal dominant diseases are those in which only one allele of the gene needs to be mutated for the disease or trait to develop. Autosomal dominant diseases can be broken down into two categories: autosomal dominant gain-of-function or dominant negative and autosomal dominant haploinsufficiency, or loss-of-function. In dominant gain-of-function (dominant negative) diseases, the mutant protein possesses a new deleterious or increased function and acts therefore as a toxic protein. In our focus area of haploinsufficiency diseases, the mutated allele generates a protein that is severely deficient in amount or activity and results in approximately 50% of normal protein expression. Haploinsufficiencies include both rare conditions as well as more common disorders. Severe haploinsufficiencies typically arise from spontaneous mutation, and thus the incidence of these diseases is not reduced by pre-conception genetic screening.

Multiple therapeutic modalities, including gene therapy, gene editing, modified mRNA, protein-based drugs, small molecules and oligonucleotides are approved or are being developed to address all types of monogenic diseases. However, most of these therapeutic approaches are focused on autosomal recessive or autosomal dominant gain-of-function (dominant negative) diseases. The nature and fundamental limitations of these modalities make them poorly suited to address the underlying genetic cause of haploinsufficiency diseases. Consequently, there has been little focus on drug development for these diseases despite a significant unmet medical need.

The table below summarizes the major categories of genetic diseases and the various precision medicine approaches that are actively being used or explored to address them.

Category	Autosomal recessive	Autosomal dominant gain-of-function / dominant negative	Autosomal dominant haploinsufficiency
Genetic mutation	Loss-of-function mutations in both gene alleles	Gain-of-function or dominant negative mutation in one gene allele (toxic protein)	Loss-of-function mutation in one gene allele
Result	Less than 10-25% of normal protein expression	Deleterious or increased protein expression	Approximately 50% of normal protein expression
Disease examples	• Phenylketonuria • Lysosomal storage disorders • Beta-thalassemia • Cystic fibrosis	• Huntington's disease • Parkinson's disease • Spinocerebellar ataxia • AD hypocalcemia	• Dravet syndrome • Optic atrophy • Tuberous sclerosis • Polycystic kidney disease
Current and emerging precision medicines	• Gene therapy • Gene editing • Modified mRNA • Protein-based drugs • Small molecules	• Gene therapy • Gene editing • Protein-based drugs • Small molecules • Oligonucleotides	• Our TANGO technology[1] STOKE THERAPEUTICS
Therapeutic goal	Upregulate protein expression to greater than 10-25% of normal	Downregulate protein expression / inhibit protein function	Upregulate protein expression to near normal

(1) We have neither applied for, nor received, FDA approval for any of our product candidates to date.

Current and emerging precision medicines and their limitations

While current and emerging precision medicine approaches have already made and will likely continue to make significant advancements, we believe they currently possess fundamental limitations which must be overcome before they will become a practical approach to treating genetic diseases, especially autosomal dominant haploinsufficiencies.

Gene therapy

Gene therapy is designed to introduce a functional copy of a defective gene or gene sequence into a patient's cell. This therapeutic approach provides the potential to replace the defective genes that lead to disease.

Today, gene therapy is subject to several technical challenges. Single stranded adeno-associated virus (ssAAV) gene therapy approaches are unable to efficiently package more than 4.4 kilobases of coding DNA and the more efficient self-complementary AAV (scAAV) are limited to 2.1 kilobases of coding DNA; thereby restricting their utility to smaller gene targets. In addition, the inability of current approaches to establish tunable control of the level of protein expression and tissue specificity raises concerns of possible unintended DNA changes and unwanted on- and off-target effects. Further, gene therapy vectors are complex delivery systems, which significantly increase the cost of manufacturing and the difficulty of maintaining reliable quality among product lots.

Gene editing

A more recent approach is gene editing, which is the process of replacing, deleting or repairing defective DNA in its native genomic location. The current focus of gene editing is knocking out a diseased gene or correcting an individual mutation within a gene that is frequent within the disease population. The approach faces many similar challenges to gene therapy and has yet to achieve clinical proof-of-concept.

Gene editing currently suffers from numerous limitations, including the inability to control level and duration of protein expression, a potential for irreversible unintended DNA changes and unwanted on- and off-target effects, and a complex delivery and manufacturing process. In addition, gene editing repairs one mutation at a time, and thus is not well-suited for the treatment of diseases caused by many mutations in a single gene, as is the case for many haploinsufficiencies, which typically result from multiple spontaneous mutations.

Modified mRNA

Over the past several years, there has been significant investment and progress in the field of modified mRNA. These therapies are designed to increase mRNA levels by exogenous delivery of modified mRNA.

However, modified mRNA is characterized by significant drug delivery hurdles and its clinical application has largely been limited to novel vaccines. This therapeutic modality also does not permit precise targeting of tissue and requires complex manufacturing processes that are unproven at commercial scale. In addition, modified mRNA requires frequent administration given its short duration and safe repeat dosing has yet to be achieved clinically. Finally, the ability to package large genes is also unproven and may limit utility to smaller gene targets.

Protein-based drugs

Protein-based drugs are manufactured in living cells and can bind with high specificity to a variety of extracellular or cell surface targets or can be used to replace mutated or missing extracellular proteins. Protein-based drugs can also bind to a very narrow spectrum of intracellular proteins (lysosomal storage proteins). Antibodies (and antibody-like proteins) have become the most common type of biologic because of the specificity and long duration of action of this type of molecule. Monoclonal antibody drugs typically act by inhibiting target proteins through competitive binding (antagonists).

Currently, protein-based drugs are unable to address most diseases caused by deficient activity of intracellular or transmembrane proteins, such as ion channels involved in genetic epilepsies. Additionally, complex manufacturing and short duration after administration can prevent maintaining therapeutic levels of the protein in the body.

Small molecules

Small molecules consist predominantly of hydrophobic organic compounds under 500 daltons in molecular weight and are manufactured through chemical synthesis. These drugs typically act by deactivating or inhibiting target proteins through competitive binding (antagonists). In much rarer instances, small molecule agonists can sometimes be identified that increase the activity of a target protein through binding to a regulatory site.

Small molecules are artificial agonists which act through non-natural mechanisms, and therefore do not fully compensate for the loss of a protein that functions in a regulated fashion or as part of a multi-protein complex. Applications for small molecules are also very limited, and proteins that possess small molecule-binding pockets have been estimated to account for only 2-5% of the human proteome. Small molecules also lack selectivity and specificity, creating the potential for off-target toxicity. Finally, these therapeutics typically do not address the underlying cause of genetic diseases and consequently may have limited impact on patient quality of life or life expectancy.

Oligonucleotides

Oligonucleotides are short strands of modified RNA or DNA, usually 12-30 nucleotides in length, that are manufactured by chemical synthesis. Single-stranded oligonucleotides that bind to mRNA are called ASOs, which have been developed primarily to downregulate protein expression by RNase H-mediated cleavage of target mRNA.

Over the past few years, there has been very limited success in developing clinical ASOs to upregulate protein expression due to a focus on indirect and weakly validated mechanisms of action such as targeting microRNAs or long non-coding RNAs that are associated with a gene transcript. The only exceptions are SPINRAZA, which corrects a unique splicing mutation in *SMN2*, and Exondys-51, which generates an internally-truncated form of dystrophin after removing or 'skipping' out a mutated exon to restore the reading frame. Neither drug represents a generalizable strategy to upregulate the expression of protein. Similarly, double-stranded oligonucleotides have been developed primarily to downregulate protein expression by RNA interference mediated cleavage of target mRNA. To date, there has been very limited success in developing clinical double-stranded oligonucleotides to upregulate protein expression.

Given these fundamental limitations of existing modalities, most genetic diseases, particularly autosomal dominant haploinsufficiencies, are dramatically underserved by current therapeutic options. Within rare diseases, only 5% of conditions have an approved drug treatment, and most approved drugs only manage symptoms with little impact on outcomes and life expectancy. We believe there is a clear need for our novel ASOs, which precisely upregulate target protein expression and have the potential to provide disease-modifying therapies to treat many diseases beyond the reach of current approaches.

Our precision medicine platform

Treatment of autosomal dominant haploinsufficiency diseases with TANGO

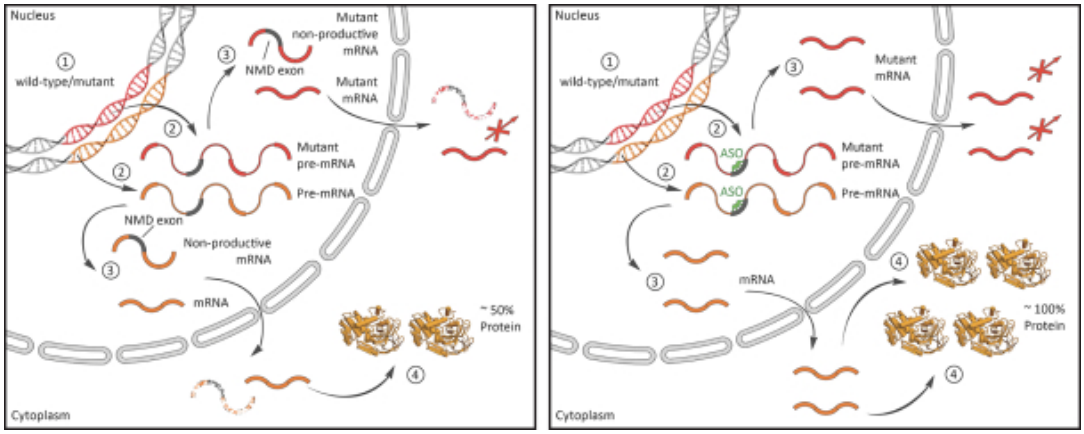
We are developing our proprietary technology platform, TANGO, as potentially the first precision medicine platform for a category of severe genetic diseases known as autosomal dominant haploinsufficiencies. We utilize TANGO to design ASOs to increase the expression of protein by individual genes in a patient. TANGO exploits unique mechanisms for modulation of splicing to prevent the synthesis of naturally occurring non-productive mRNA and increase the synthesis of productive mRNA, resulting in increased production of functional protein.

TANGO leverages non-productive mRNA, which is the result of non-productive splicing that leads to either transcript degradation due to non-coding exon inclusion or nuclear retention of transcripts due to intron retention. In some cases, these non-productive splicing events are a part of normal gene regulation, and in all cases the non-productive splicing events are part of the wild-type or normal sequence of the gene. Non-productive mRNA can be produced by both wild-type and mutant alleles and is not translated into protein.

We are initially focused on applying the transformative potential of our platform to developing precision medicines for haploinsufficiencies, or disorders in which only one allele of a gene is mutated, resulting in approximately 50% of normal protein expression. We believe our TANGO technology is well-suited to provide a gene-specific increase in expression of the healthy, or wild-type, allele, thereby restoring the target protein to near normal levels.

The figures below illustrate the TANGO mechanism for increasing protein synthesis in a prospective patient with a haploinsufficiency. To date, we have demonstrated this TANGO mechanism in preclinical models of haploinsufficiencies. The left panel illustrates the prospective patient with a haploinsufficiency possessing one wild-type allele and one mutant allele. The mutant allele is translated into non-functional protein and results in

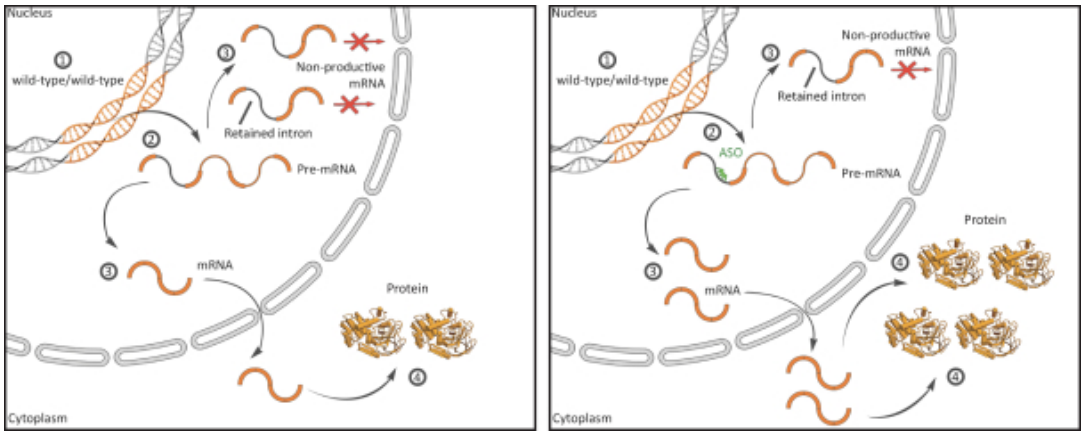
approximately 50% of normal protein expression. In the right panel, treatment with our ASO would prevent the synthesis of naturally occurring non-productive mRNA and would increase the synthesis of productive mRNA, thereby restoring the target protein to near normal levels. Our preclinical studies show that any increase in mutant mRNA would have no effect on the net protein level.



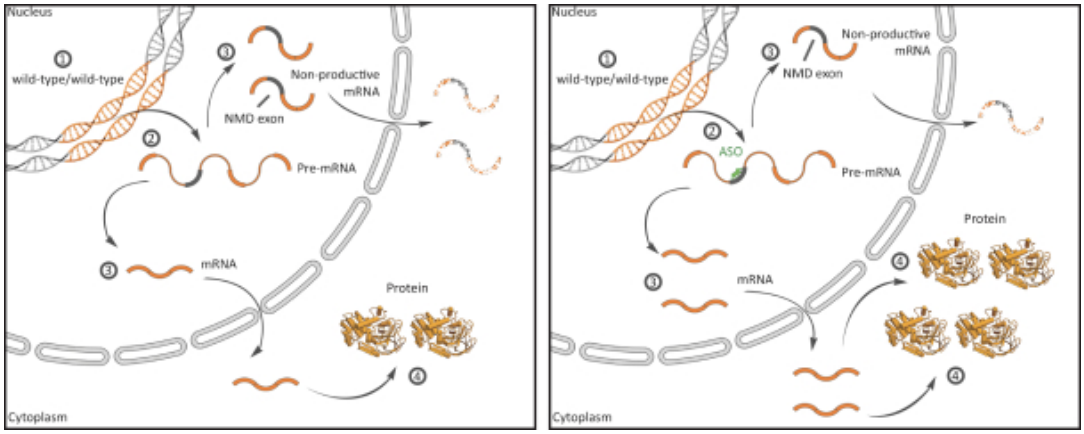
TANGO mechanisms of action

Our ASOs are specifically designed to bind to a desired RNA sequence inside the nuclei of patients' cells to prevent the occurrence of non-productive splicing. By doing so, our ASOs decrease the amount of non-productive mRNA and increase the level of productive mRNA, leading to the generation of more protein. TANGO operates in a mutation-independent manner, given it utilizes one wild-type allele, and does not alter protein coding splicing isoforms. The net effect is increased expression of functional protein from the wild-type allele. The two categories of non-productive splicing events amenable to TANGO are retained introns and nonsense-mediated mRNA decay of the resulting mRNA. While we benefit from leveraging previously-validated ASO chemistries, both of these TANGO mechanisms are novel.

The first category of non-productive splicing events amenable to TANGO is retained introns. Retained introns are found in approximately 60% of gene transcripts and are part of the wild-type sequence of the gene. In some cases, retained introns are part of normal gene regulation. The non-productive mRNA, which contains these retained introns, remain in the nucleus of the cell and are not translated into protein, and offer a reservoir of non-productive mRNA that can be converted into productive mRNA. Our ASOs bind to the pre-mRNA and redirect the splicing machinery to remove the retained intron. This splice-switching decreases non-productive mRNA and increases productive mRNA, which is translated into increased protein expression from the wild-type allele. This is shown in the figures below, with the left panel illustrating non-productive mRNA, which includes retained introns, and the right panel illustrating our ASOs binding to the pre-mRNA and redirecting the splicing machinery.



The second category of non-productive splicing events amenable to TANGO is alternative splicing that leads to nonsense-mediated mRNA decay, or NMD, of the resulting mRNA. An example of a NMD event is a NMD exon, which is found in over 25% of gene transcripts. Like retained introns, NMD exons are part of the wild-type sequence of the genes. In some cases, NMD exons are part of normal gene regulation. Non-productive mRNA, which includes these NMD exons, is degraded in the cytoplasm of the cell by nonsense-mediated mRNA decay and is not translated into protein. Our ASOs bind to the pre-mRNA and redirect the splicing machinery to prevent inclusion of the NMD exon. As with retained introns, this splice-switching decreases non-productive mRNA and increases productive mRNA, which is translated into increased protein expression from the wild-type allele. In contrast to current exon skipping therapies, which remove a coding exon and result in a truncated protein, our TANGO mechanism skips out a non-coding NMD exon and yields a full-length functional protein. Our lead product candidate, STK-001, targets an NMD exon and the general mechanism is shown in the figure below, with the left panel showing the non-productive mRNA failing to be translated into protein and the right panel showing our ASOs binding to the pre-mRNA and redirecting the splicing machinery. Although STK-001 is currently our only current product candidate and is still in preclinical testing, we would expect, based on our preclinical studies, for future product candidates targeting a NMD exon to operate in the same manner.



Advantages of TANGO

We believe TANGO may have several key advantages, including:

- *Ability to address the underlying genetic cause of the disease.* We utilize TANGO to design ASOs to precisely upregulate protein expression, thereby addressing the underlying cause of the disease rather than the symptoms of the disease.
- *Applicability is mutation-independent.* Our ASOs upregulate expression of the wild-type allele, meaning the TANGO mechanism does not rely on targeting a specific mutation. Given this, we believe our therapies are well-suited for diseases caused by multiple mutations in a single gene, such as many haploinsufficiencies, and provide a single-drug approach that can address the full spectrum of loss-of-function mutations.
- *Utility across small and large gene targets encoding intracellular and extracellular proteins.* Our ASOs upregulate protein expression regardless of gene size and are not constrained to smaller gene targets. We believe our therapies also have the flexibility to address genes encoding intracellular as well as extracellular proteins.
- *No observed unwanted off-target effects.* Our ASOs do not create detectable changes at the DNA level and make no detectable irreversible modifications to a patient's genome. The activities of our ASOs are inherently tissue-specific. TANGO-mediated upregulation of protein expression only occurs where the gene is being naturally transcribed, limiting the likelihood of expression in non-native tissues.
- *Ability to control dose level and duration.* Our ASOs provide the ability for dose titration, thereby allowing for dose-dependent and reversible control of level and duration of protein expression. The ability to titrate dosage provides us with flexibility to address a variety of tissue types, and potentially enables us to deliver the right dose, at the right location, for each indication.
- *Utility across wide array of diseases and tissue types.* We believe that ASO delivery to the CNS, eye, kidney and liver is well-established, providing us the potential to address a broad range of genetic diseases. Additionally, although FDA approval for any of our current or future product candidates is not assured, we believe that ASO delivery to the CNS is particularly well-precedented.
- *Fixed dose, rather than weight-based dosing*. We have observed that while the quantity of non-productive mRNA can vary across tissue types for a gene, it remains constant across individuals. As a result, for CNS and eye targets, the dose of our ASOs should not require adjustment between patients to be effective. We believe that a fixed dose across all ages in these targets will lessen reimbursement hurdles associated with a weight-adjusted dose pricing model.
- *Favorable dosing regimen.* We believe our ASOs may require as few as two to three administrations per year for the CNS or the eye and will generally involve relatively low doses, which would translate to simplified use, an improved safety profile from reduced systemic exposure and lower cost of goods.
- *Simple and scalable manufacturing*. Our novel ASOs are synthesized by highly scalable, solid-phase chemical synthesis and we leverage a well-established contract manufacturing base. We believe the manufacturing requirements for our ASOs are much simpler, more scalable and more cost-effective than gene therapy and gene editing.

Our approach

We employ a systematic and capital-efficient approach to develop ASOs for genetically defined patient populations. We rely on our proprietary database to identify novel drug targets and corroborate these findings with existing knowledge to improve our probability of success in the clinic. We believe that leveraging our proprietary database and focusing on our core competencies of target identification and clinical and regulatory execution will allow us to reduce the time, cost and risks of drug development.

Target identification

We continue to make significant investments in our infrastructure to accelerate the pace and scale of target identification. We have built a significant bioinformatics capability, which includes proprietary bioinformatics algorithms and extensive in-house expertise in whole transcriptome RNA sequencing, also referred to as RNAseq. RNAseq uses next-generation sequencing to determine the quantity and sequences of RNA in a sample. We leverage large internal datasets of RNAseq from key tissues known to be addressable with antisense, such as the CNS, eye, liver and kidney, that are purpose-built to enhance the capture of non-productive events.

We employ machine learning to iteratively refine our search and scoring criteria for the most addressable non-productive mRNA elements based on internal target validation and Hit identification data. To date, we have identified and assembled a proprietary database of approximately 85,000 non-productive events in the human transcriptome. Using this large internal data set, in combination with publicly available genetic disease databases, we have identified approximately 2,900 monogenic disease-associated genes with one or more non-productive events which we believe are amenable to our TANGO technology. We believe our approach is highly predictive and enables rapid and systematic identification of those targets that are most likely to have clinical relevance, thereby increasing the probability for clinical success and accelerating the expansion of our emerging pipeline.

Hit identification

Once a TANGO target is validated in cells and tissue that are relevant to the disease, we employ highly-efficient cell lines to rapidly screen for Hit ASOs that can increase the target protein expression by specifically preventing the occurrence of the non-productive event in the target mRNA. ASO arrays are typically 25-50 compounds per non-productive event and utilize clinically translatable previously-validated ASO chemistries, such as 2’ methoxyethyl phosphorothioate and PMO. Hit compounds are evaluated in wild-type animal models to identify Lead ASOs that possess suitable efficacy and safety to merit preclinical development. Lead ASOs are subsequently evaluated in animal disease models or *ex vivo* disease model systems.

Lead evaluation and prioritization

After we have identified lead compounds, we evaluate and prioritize the advancement of new development candidates based on both program-specific and portfolio-wide considerations. Program-specific criteria include, among other relevant factors, the severity of the unmet medical need, the likelihood of therapeutic utility, the feasibility of clinical development, the costs of development and the commercial opportunity. Portfolio-wide considerations include the ability to demonstrate technical success for our platform, thereby increasing the probability of success and learnings for subsequent programs. We believe that the learnings from our lead Dravet syndrome program will significantly reduce the uncertainty of development of subsequent programs in our pipeline, particularly those targeting the CNS.

Clinical trial and regulatory execution

We employ a multi-pronged approach to bring new treatments forward as rapidly as possible. Our approach leverages previously-validated ASO chemistry and a modality that has been successfully utilized for other diseases, to minimize potential safety concerns and development risk. We are also initially targeting diseases with established clinical and regulatory pathways. As an example, we intend to undertake a Phase 1/2 clinical trial for our lead program in Dravet syndrome with a design and endpoints common to other recently approved antiepileptic drugs. Additionally, we plan to begin clinical dosing in patients at a dose anticipated to have biological effect to allow for early insight into the therapeutic potential of a product candidate and the possibility for rapid clinical development and expedited regulatory pathways, in addition to Fast Track Designation and Breakthrough Therapy Designation.

Commercialization

We intend to retain broad commercial rights and independently bring our therapies to patients around the world through a lean, targeted internal commercial organization. To do this, we are focused on ensuring that

we can effectively identify and access those patients who will benefit from our therapies. We target diseases in which genetic testing is routinely performed, thereby shortening the diagnostic odyssey and enabling rapid identification of patients who harbor the relevant genetic mutations. We have partnered with Invitae, a leading genetic information company, to provide genetic testing at no cost to the patient. Lastly, to maximize patient access, we aim to leverage an established network of academic and tertiary centers with extensive experience with analogous drug administration.

Therapeutic focus and product candidates

We believe our ASOs can be applied to treat a wide range of severe genetic diseases, and we have carefully designed and prioritized our pipeline strategy to maximize this opportunity. We are focused on applying the transformative potential of our platform to developing medicines for patients with diseases where the genetic abnormality is known and is found in a single gene. We therefore know for a given disease precisely which gene will need to be upregulated, thus mitigating against the uncertainty of the disease biology. We are currently focused on developing product candidates to treat autosomal dominant haploinsufficiency diseases, or disorders in which one copy of a gene is mutated and results in approximately 50% of normal protein expression. Within haploinsufficiencies, we believe that no other disease area holds as clear a need or as much promise for near-term medical breakthrough as genetic epilepsies, including Dravet syndrome, and therefore we are prioritizing this disease area for our near-term development efforts.

Genetic epilepsies

Epilepsy is defined as recurrent, unprovoked seizures due to abnormal, asynchronized neuronal firing in the brain. Epilepsy is the fourth most common neurologic disease and affects more than 50 million people worldwide, according to the World Health Organization as of 2019. Epilepsy is the most frequent serious chronic neurologic condition in childhood, and approximately one out of 150 children are diagnosed with epilepsy during the first 10 years of life, with the highest incidence rate observed during infancy, according to a 2017 publication in *Pediatrics*.

More than 30% of patients with epilepsy are refractory to medical treatment, especially those with a genetic epilepsy, despite the availability of approximately 20 antiepileptic drugs. This is largely because existing antiepileptic drugs primarily address the frequency of seizures and lack the capacity to rectify the underlying neuropathological processes or genetic defect. Refractory epilepsy carries the risks of structural damage to the brain and nervous system and increased risk of premature death (e.g. from sudden unexpected death in epilepsy, or SUDEP, suicide, accidents, pneumonia, or vascular disease), as well as psychological, educational, social and vocational consequences. In addition, up to 50% of patients with epilepsy have significant cognitive delay, according to a 2015 review article in *Cold Spring Harbor Perspectives*. Cognitive and behavioral comorbidities are especially common in children with refractory epilepsy. Overall outcomes in patients with epilepsy have not improved significantly over the past two decades, and a novel therapeutic approach is desperately needed to modify the development or progression of the disease and improve long-term outcomes.

A 2010 publication in *Nature Reviews Neurology* estimates that more than 50% of epilepsies are now recognized as having a genetic basis, and many of these are haploinsufficiencies. The genetic bases of both rare and common epilepsies are rapidly being elucidated, and neurologists now routinely include genetic testing for more than 180 disease-associated genes in the diagnostic work-up of epilepsy. Beyond diagnostics, a major goal of genetic testing is to enable individualized treatment choices based on the genetic cause of disease. Today, the application of genetic diagnosis for epilepsy patients is largely limited to medical contraindications, such as avoidance or removal of ion channel blockers for an ion channel deficiency, given that there are no genetically-targeted medicines available for genetic epilepsy patients.

Several hundred epilepsy-related genes have been identified to date, including genes encoding neuronal ion channels and receptors and genes involved in cellular signaling. For example, the number of genes included on

the epilepsy panel of Invitae Corporation, a leading genetic information company, has grown from 103 in 2015 to 187 in 2019. Globally, advances in molecular technology are expected to result in discoveries of additional genetic etiologies of epilepsy, implying a greater role than ever before for genetics in the epilepsy clinic.

The table below lists select genes that are commonly mutated amongst patients with epilepsy and we believe are amenable to TANGO, with current estimates of their prevalence. We are continuing to evaluate these CNS targets and expect to nominate a second genetic disease candidate for preclinical development by the first half of 2020.

Gene	Disease	Estimated worldwide prevalence
SCN1A	Dravet Syndrome	5-5.5 in 100,000
TSC2	Tuberous Sclerosis 2	7-8 in 100,000
MECP2	Rett Syndrome	5 in 100,000
TSC1	Tuberous Sclerosis 1	2-3 in 100,000
SCN2A	Epileptic Encephalopathy	1-2 in 100,000
CHD2	CHD2-Myoclonic Epilepsy	1-2 in 100,000
SYNGAP1	Autosomal Dominant Mental Retardation 5	1 in 100,000
SCL6A1	Epileptic Encephalopathy	1 in 100,000
SCN8A	Epileptic Encephalopathy	1 in 100,000
CACNA1A	Episodic Ataxia, Type 2	<1 in 100,000

For some genes, the phenotypic spectrum expands beyond the epilepsies to other neurodevelopmental disorders, including autism and intellectual disability. For example, although most patients with mutations in *STXBP1*, *SYNGAP1* or *CHD2* present with seizures, mutations in these genes have also been identified in individuals with intellectual disability or autism spectrum disorder, but without epilepsy. These neurodevelopmental disorders are not addressed with existing antiepileptic drugs.

Dravet syndrome—STK-001

Our most advanced program is a potentially disease-modifying treatment for Dravet syndrome, a severe and progressive genetic epilepsy. STK-001 is a proprietary ASO and utilizes an established delivery mechanism of intrathecal delivery to target the CNS. We applied for Orphan Drug Designation from the FDA in May 2019, and intend to submit an IND by early 2020. We expect to initiate a Phase 1/2 clinical trial in children and adolescents with Dravet syndrome in the first half of 2020 and anticipate clinical data, including preliminary efficacy data, in 2021. If we see evidence of efficacy following clinical data, then we would plan to meet with regulatory authorities to discuss expedited regulatory pathways, in addition to requesting Fast Track Designation and Breakthrough Therapy Designation. To date, the FDA has given no indication to whether our product candidate will receive Orphan Drug Designation or be permitted to use any such expedited pathway.

Dravet syndrome disease overview

Dravet syndrome is one of the most severe genetic epilepsies and affects approximately 6.4 in 100,000 people worldwide, including 5-5.5 in 100,000 people who possess a mutation in the *SCN1A* gene, according to a 2018 market research report commissioned by us and prepared by Health Advances, LLC, or the Health Advances Report. The disease is caused by a pathogenic mutation or deletion of the *SCN1A* gene in approximately 85% of patients. At least 1,250 different *de novo* mutations in the *SCN1A* gene have been identified to date in Dravet syndrome patients, including single nucleotide substitutions, small insertions or deletions and even whole gene deletions. *SCN1A* codes for the alpha subunit of the voltage-gated sodium channel, or $Na_v1.1$ protein, an ion channel that is essential for the generation and propagation of action potentials. More than 95% of the disease-causing mutations of *SCN1A* cause loss-of-function, resulting in haploinsufficiency (approximately 50%

reduction) of the $Na_v1.1$ protein in select neurons in the brain. This loss of $Na_v1.1$ channels in inhibitory interneurons and other nerve cells results in Dravet syndrome.

Dravet syndrome is characterized by multiple seizure types and may progress to status epilepticus or prolonged seizures lasting more than five minutes that require immediate intervention. Patients typically experience their first seizure before 12 months of age. More than 90% of patients suffer from at least one non-seizure comorbidity, including severe intellectual and developmental disabilities, motor and speech impairment, autism, attention deficit hyperactivity disorder and behavioral difficulties. Neurologic function and cognition are usually normal in children with Dravet syndrome up to two years of age. However, nearly all Dravet syndrome patients exhibit intellectual impairment by the age of four, ranging from minor learning difficulty to global developmental delay. The time between one year and eight years of age is a critical period for intervention. After eight years of age, nearly all Dravet syndrome patients exhibit evidence of substantial developmental delay. The symptoms of the disease result in remarkably low quality of life and shortened life expectancy, and as a result impose an immense burden on individuals and families.

The cognitive impairment in Dravet syndrome is not purely a consequence of seizures. Patients with few seizures have been observed to possess severe encephalopathy, and conversely patients with frequent seizures have been observed to exhibit relatively minimal cognitive decline. In addition, there does not appear to be a correlation between cognitive outcome and *SCN1A* mutation type, whether a missense or truncating mutation.

Importantly, patients with Dravet syndrome have an increased risk of premature death, primarily due to SUDEP. Dravet syndrome patients have the highest SUDEP rate of any epilepsy. An analysis of mortality in the Epilepsy Genetics Research Program demonstrated a Dravet syndrome-specific mortality rate of 15.84 per 1,000 patient years. SUDEP was the most common cause of premature death among Dravet syndrome patients (59%), equating to a Dravet syndrome-specific SUDEP rate of 9.32 per 1,000 patient-years. This is nearly twice the rate for adults with refractory epilepsy.

Patients with Dravet syndrome are often diagnosed by three years of age, and neither patient gender nor family history of seizures is associated with risk of Dravet syndrome. Dravet syndrome occurs worldwide and is not concentrated in any particular geographic area or ethnic group. Early diagnosis is driven by heightened awareness of Dravet syndrome and other genetic epilepsy disorders as well as an emerging consensus amongst epilepsy specialists that early diagnosis is cost-effective and beneficial for prognosis. Among pediatric Dravet syndrome patients, approximately 60% in North America and 70% in Germany, France and the United Kingdom undergo genetic testing as part of their diagnostic work-up, according to the Health Advances Report. We expect this to increase to approximately 85% in North America, Japan, Germany, France and the United Kingdom by 2024 in the aggregate. The incidence of Dravet syndrome is approximately 64 per million births, which translates to an overall prevalence of approximately 35,000 patients across the United States, Canada, Japan, Germany, France and the United Kingdom, with approximately 16,000 patients in the United States. By comparison, the prevalence of spinal muscular atrophy is approximately 10,000 patients in the United States.

Current treatments

Current treatments for Dravet syndrome only address the occurrence of seizures, not the underlying cause, and according to a 2017 study as published in the *Developmental Medicine & Child Neurology* Journal, more than 90% of Dravet syndrome patients still report suffering from incomplete seizure control with existing antiepileptic regimens. As a result, the current treatment strategy involves the use of multiple antiepileptic drugs, including combinations of cannabidiol, stiripentol, clobazam, valproate, topiramate and others. Patients are typically treated with two to four drugs administered concomitantly, and in most cases the relief provided by polytherapy is insufficient.

Cannabidiol (Epidiolex) and stiripentol (Diacomit) are currently the only FDA-approved antiepileptic drugs for the treatment of Dravet syndrome. Cannabidiol was approved in 2018 for the adjunctive treatment of seizures associated with Lennox-Gastaut syndrome and Dravet syndrome in patients two years of age and older.

Diacomit was approved in 2018 for the treatment of seizures associated with Dravet syndrome in patients two years of age and older taking clobazam. There are no clinical data to support the use of Diacomit as monotherapy in Dravet syndrome. These new therapies were approved based on a demonstrated reduction in seizure frequency; however, very few patients had complete control of seizure activity. For patients treated with Epidiolex, only 6.7% reported no convulsive seizures during the treatment period, according to clinical trial data in the drug's prescribing information. Tolerance, or a significant diminishment of efficacy over time, is also observed in approximately 25% of patients, thereby limiting the usefulness of this treatment in the long-term clinical management of patients with Dravet syndrome. These drugs also do not address the significant non-seizure comorbidities. Additionally, cannabinoids as a drug class have been associated with adverse effects on cognitive development in children.

Fenfluramine (Fintepla) is an antiepileptic drug in clinical development for the treatment of Dravet syndrome. Topline efficacy data for seizure frequency were favorable; however, as with Epidiolex and Diacomit, seizure-free rates remain in the low single digits. Moreover, patients are still likely to be affected by non-seizure comorbidities and may develop tolerance to the drug over time.

Many of the antiepileptic drugs that are used to treat Dravet syndrome can carry a substantial adverse event burden. Some of the adverse events can cause deleterious effects on cognition and lead to sedation, somnolence, inattention and fatigue, and potentially lead to death. These adverse effects may exacerbate the underlying cognitive deficits that are part of the natural course of Dravet syndrome. Patients often require regular office visits and laboratory testing to monitor toxicity to vital organ systems, especially the liver, which may be further compounded by polytherapy and associated drug-drug interactions. Despite these risks, the continued use of these medications demonstrates the importance of reducing the frequency of seizures to the patients, caregivers and the prescribing neurologists.

Patients with Dravet syndrome need a novel therapeutic that addresses the genetic basis of the disease and treats the large number of seizures and multiple seizure types that persist despite treatment with existing therapy. Importantly, additional therapy options are needed to address the disabling comorbidities that occur with Dravet syndrome. If STK-001 is approved by the FDA, we believe our precision medicine approach may have a profound impact on individuals and families.

STK-001: Product candidate

We believe that STK-001 has the potential to be the first disease-modifying therapy to address the genetic cause of Dravet syndrome by restoring physiological $Na_v1.1$ levels and reducing both occurrence of seizures and significant non-seizure comorbidities.

STK-001: Preclinical data

We have generated compelling preclinical data that we believe demonstrates proof-of-mechanism for STK-001. Our initial target engagement, pharmacology and efficacy studies were performed in mice, including both wild-type and a Dravet syndrome mouse model. The Dravet syndrome mouse model replicates many of the symptoms of Dravet syndrome patients, and the targeted non-productive splicing event in *SCN1A* is highly conserved across multiple species, including mouse, non-human primates and humans. The target sequence for STK-001 is also identical across species.

In wild-type mice, we characterized target engagement and pharmacology of STK-001. Five groups of neonate (postnatal day one) mice were administered a single injection dose of 0 (n=5), 0.3, (n=5) 1.0. (n=6) 3.0 (n=3) and 10.0 (n=5) µg of STK-001 by intracerebroventricular injection and returned to the home cage for five days. Sections of the brain were processed for RNA and protein. We observed that treatment with STK-001 resulted in a dose-dependent reduction of non-productive mRNA. Furthermore, the reduction of non-productive mRNA was associated with an increase of productive mRNA and an increase in $Na_v1.1$ protein, as denoted in the figures below.

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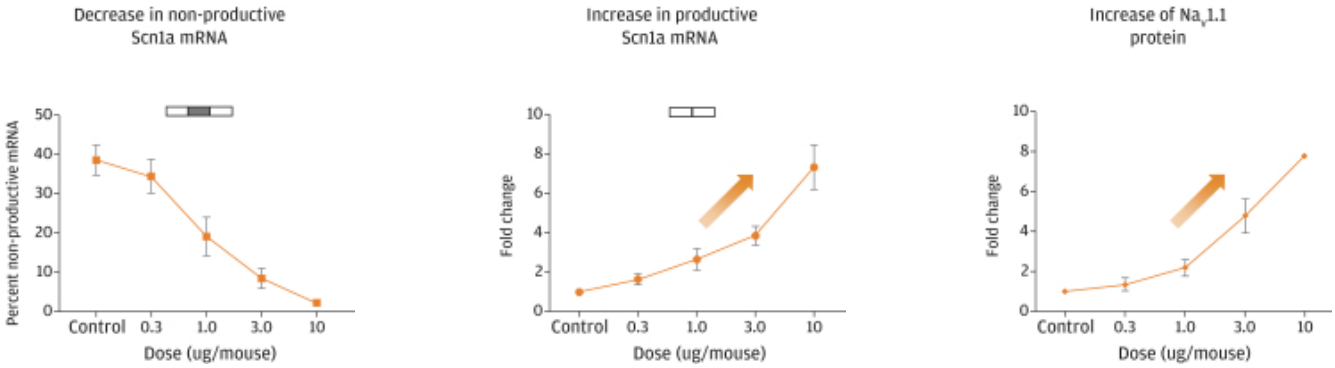
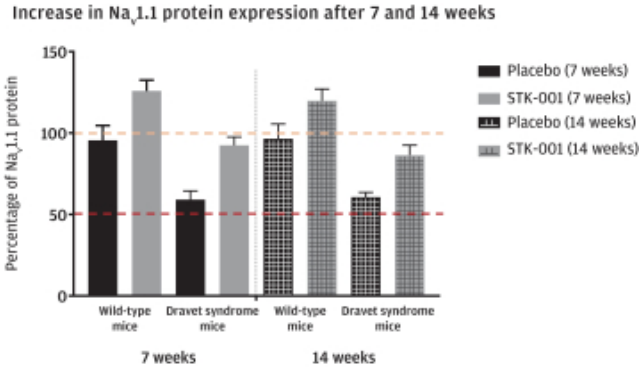
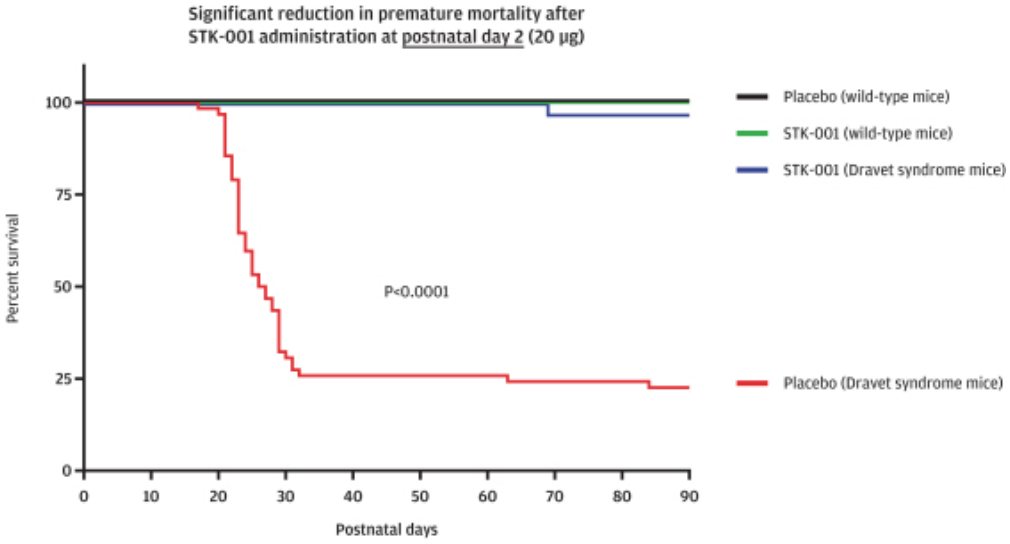


We also evaluated STK-001 pharmacology and efficacy in transgenic mice with a heterozygous deletion of *Scn1a*. This model was created by introducing a targeted deletion in the first coding exon of the *Scn1a* gene; these mice exhibit many aspects of the Dravet syndrome phenotype including seizures and premature lethality.

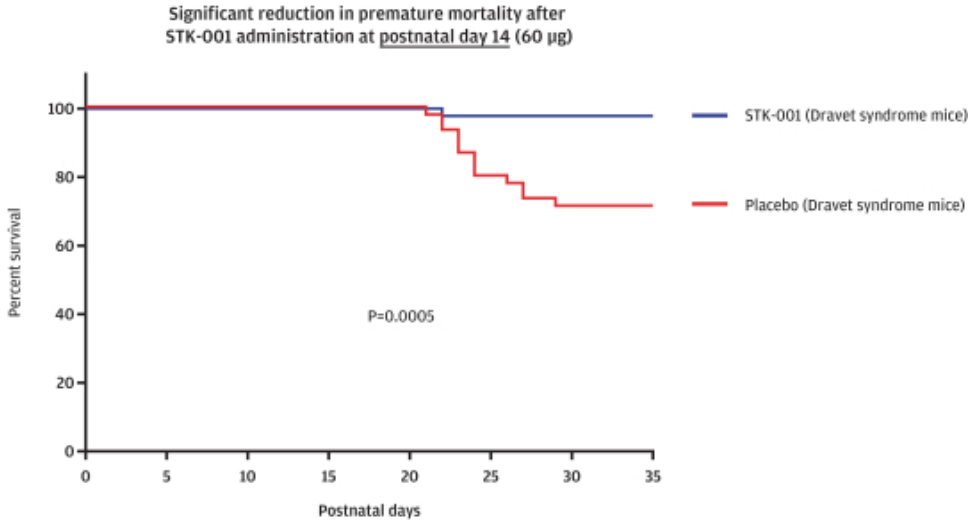
Neonate (postnatal day two) Dravet syndrome mice and wild-type littermate controls were administered a single dose of either placebo (consisting of a phosphate-buffered solution), or 20 µg of STK-001 (n=~50/group) by intracerebroventricular injection. Animals from each group were monitored through day 90. Brains were collected from cohorts of these animals at approximately 7 weeks after dosing (placebo: n=11 wild-type mice, n=4 Dravet syndrome mice; STK-001: n=9 wild-type mice, n=10 Dravet syndrome mice) and 14 weeks after dosing (placebo: n=10 wild-type mice, n=10 Dravet syndrome mice; STK-001: n=10 wild-type mice, n=10 Dravet syndrome mice). Notably, a single injection of STK-001 restored $Na_v1.1$ protein in Dravet syndrome mice to levels that are near those of the wild-type mice at both 7 and 14 weeks. These data demonstrate that STK-001 has an impact on $Na_v1.1$ protein expression and we believe this will translate to a favorable dosing regimen in humans.



In addition to an increase in the $Na_v1.1$ protein, the administration of a single dose of 20 µg of STK-001 in neonate Dravet syndrome mice (postnatal day two) resulted in a significant reduction in premature mortality. Treatment with STK-001 resulted in 97% survival of Dravet syndrome mice for the 90-day post-natal observation period (survival of 33 out of 34 mice was observed in the STK-001 Dravet syndrome mouse group) compared with 23% survival of placebo-treated mice (survival of 14 out of 62 mice). This is illustrated in the figure below.

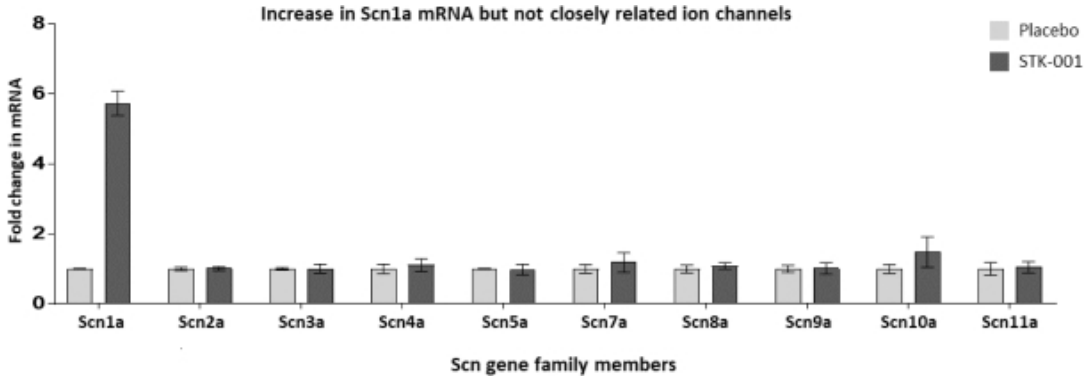


In additional studies, Dravet syndrome mice were treated closer to symptom onset (postnatal day 14). An interim analysis through postnatal day 35 indicated a significant reduction in premature mortality. Treatment with a single dose of 60 µg of STK-001 resulted in 98% survival of Dravet syndrome mice for the 35-day post-natal observation period (survival of 45 out of 46 mice was observed in the STK-001 Dravet syndrome mouse group) compared with 71% survival of placebo mice (survival of 32 out of 45 mice). This is illustrated in the figure below.



Analyses were also performed *in silico* to understand the specificity of STK-001. We evaluated STK-001 via bioinformatic analysis against all annotated protein-coding genes to predict potential off-target activities. Results showed no perfect 18- to 16-nucleotide match for STK-001 anywhere in the transcriptome other than *SCN1A* pre-mRNA, indicating that STK-001 recognizes a unique sequence in the human transcriptome and should possess minimal off-target bindings.

Further supporting our specificity analysis, we also evaluated brain samples of wild-type neonate mice to ensure that STK-001 does not alter levels of other channels in the highly homologous *SCN* family. Importantly, the mRNA levels of closely related ion channels was not altered in the mouse brain five days after administration of 10 µg of STK-001 (n=2/group placebo, n=4/group STK-001), as shown in the figure below. Similar analysis was performed in wild-type and Dravet syndrome mice treated with 20 µg of STK-001 at 7 and 14 weeks after dosing. STK-001 treated samples showed an increase in expression of the *SCN1A* gene, but not any of the other *SCN* family members. These biological studies demonstrate that STK-001 is highly specific for *SCN1A* among the highly homologous family of sodium channel genes, limiting the likelihood of off-target activities.



We also investigated the pharmacology, distribution and tolerability of STK-001 in a study with cynomolgus monkeys. As a pilot experiment, this study was not required to be performed under Good Laboratory Practices, or GLP. Pre-pubescent monkeys (age 2-2.5 years old) were administered a single dose of STK-001 (n=3/group; 4 groups dosed) or control solution (n=2/group; 2 groups dosed) by intrathecal injection at a dose range that we believe coincides with the estimated therapeutic dose range and stays below the maximum tolerated dose based on tolerability in mice and published data for molecules of similar chemistry. The animals were sacrificed at 3 days (n=8) and 29 days (n=8) after dosing. An increase in $Na_V1.1$ levels was observed ranging from 1.1-fold to 2.0-fold, compared to the control group, varying by the anatomical region, dose and day of necropsy, with the greatest changes observed in the cerebral cortex. The increase in $Na_V1.1$ was also correlated with the presence of STK-001 in brain tissue. Additionally, all doses tested showed no drug-related toxicities, including no changes in platelet counts or hepatic function, no clinical signs or symptoms over the 28-day period after administration and no abnormal histopathology.

While the non-GLP study described above will be included in our IND application, further studies in rats and cynomolgus monkeys, including GLP single and multiple dose toxicology studies, are planned to further characterize the pharmacology, exposure and tolerability of STK-001 and will be necessary to support an IND.

STK-001: Clinical plan

We expect our Phase 1/2 trial to be a two-part study to evaluate STK-001 in children and adolescents with Dravet syndrome. Patients will be eligible for the trial if they are between the ages of 2 to 18, have had four or more convulsive seizures during a four-week pre-dosing observation period, have an established diagnosis of

Dravet syndrome and have evidence of a pathogenic genetic mutation in the *SCN1A* gene. Requiring an *SCN1A* mutation (of which more than 1,250 *SCN1A* mutations have been identified) for trial enrollment allows for a clear and definitive etiologic diagnosis, a more homogeneous patient population and tailored treatment based on a precision medicine approach. Eligible patients will also have failed at least two epilepsy treatments in the past and currently be taking at least one antiepileptic drug. All medications and interventions will remain unchanged throughout the trial, which will allow for assessment of STK-001 with a variety of antiepileptic therapies.

The trial will be conducted in two parts: single ascending dose and multiple ascending dose. The primary objectives will be to assess the safety and tolerability of STK-001, as well as to characterize human pharmacokinetics. A secondary objective will be to assess the efficacy as an adjunctive antiepileptic treatment with respect to the percentage change from baseline in convulsive seizure frequency over 12-week treatment period. We also intend to measure non-seizure aspects of the disease, such as cognitive function and quality of life as secondary endpoints. These endpoints as well as other exploratory endpoints will be informed based on our planned observational study, which is designed to evaluate the course of neurodevelopmental status and adaptive status, quality of life, gait and ambulation in patients with Dravet syndrome over a timespan of two years. To help identify patients eligible for our studies, we have an ongoing partnership with Invitae Corporation to offer epilepsy panel testing at no cost to any child up to 60 months who has had an unprovoked seizure. We expect to enroll approximately 36 children and adolescents in this observational study, and these patients will be eligible for enrollment in our Phase 1/2 clinical trial.

Importantly, recently approved antiepileptic drugs for Dravet syndrome, such as Epidiolex, provide a potential regulatory pathway to approval on defined seizure control endpoints. We will be leveraging an analogous trial design, including a four-week pre-dosing observation period to assess baseline seizure frequency, cognitive function and serum chemistries, a 12-week treatment period and a 6-month safety follow-up. We plan to begin clinical dosing at the minimum anticipated biological effect level given the limited treatment options for this patient population, known pharmacology and mechanism of action of STK-001, and reasonable confidence in the predictive value of the preclinical data generated in non-human primates. Dosing by intrathecal injections will be fixed across the patient population given that the quantity of non-productive mRNA remains constant across individuals and that the total cerebral spinal fluid volume is similar between adults and children.

We plan to submit an IND for STK-001 by early 2020. We expect to initiate the Phase 1/2 trial in the first half of 2020 and we anticipate preliminary clinical data for the primary and secondary endpoints of our single ascending dose phase in 2021. While we have not yet discussed with regulatory authorities the evidence necessary for approval of STK-001, if we see evidence of efficacy following clinical data, then we would plan to meet with regulatory authorities to discuss expedited regulatory pathways, in addition to Fast Track Designation and Breakthrough Therapy Designation.

Additional product opportunities

Dravet syndrome and genetic epilepsies represent one disease area within a broader spectrum of novel precision medicines for treatment of haploinsufficiency diseases. We intend to nominate a second genetic disease preclinical candidate by the first half of 2020 and will seek to further establish a pipeline of product candidates in the future. Since ASOs have been previously shown to have a very long half-life when injected into the eye and could provide therapeutics with dosing regimens of two to three administrations per year, we are exploring certain ophthalmologic diseases that could be treated through upregulation of protein pathways to reduce inflammation, block neovascularization and reduce retinal degeneration.

We are also advancing several early programs focused on multiple targets, including haploinsufficiency diseases of the CNS, eye, kidney and liver, given the ability of our ASOs to target cells in these organs. These tissues are affected in many severe genetic diseases. Additional non-epilepsy indications for which our

technology may be applicable include autosomal dominant optic atrophy and autosomal dominant polycystic kidney disease.

Longer-term, we believe that ASOs designed using TANGO may have the potential to upregulate non-mutated genes in biological pathways to treat diseases or conditions caused by multiple genes or are multifactorial, such as autoimmune diseases, aging and cancer. For these diseases, we intend to opportunistically secure partnerships with pharma partners whose scientific, development or commercial capabilities complement our own.

Manufacturing

We currently contract with third parties to manufacture our products undergoing late-preclinical testing and anticipate using third parties for all commercial manufacturing. We do not own or operate facilities for product manufacturing, packaging, storage and distribution, or testing. We have personnel with extensive technical, manufacturing, analytical and quality experience and good project management to oversee contract manufacturing and testing activities. We will continue to expand and strengthen our network of third-party providers but may also consider investing in internal manufacturing capabilities in the future if there is a technical need, or a strategic or financial benefit.

Manufacturing is subject to extensive regulations that impose procedural and documentation requirements. At a minimum these regulations govern record keeping, manufacturing processes and controls, personnel, quality control and quality assurance. Our systems and contractors are required to be in compliance with these regulations and are assessed through regular monitoring and formal audits.

Drug substance

Oligonucleotide drug substance requirements for our most advanced programs can be readily met by a variety of domestic and international contractors. Many of these contractors are also able to source all the required raw materials, which allows us to consolidate raw material procurement and drug substance manufacturing activities with a single supplier. To ensure supply chain continuity, we plan to establish supply agreements with alternative suppliers as appropriate. As part of each development program, efforts will be made to invest in process changes to improve purity and yield as warranted.

Future drug substance compositions may require different manufacturing capabilities, which will be addressed through either expanded capability with existing contractors or establishing manufacturing supply relationships with new contractors. These changes in composition may also require new supply chain agreements with contractors that specialize in raw material manufacturing. Our internal personnel will work to identify and establish relationships with contractors that may be ideally suited to meeting these new manufacturing requirements.

Drug product

In the near future, we expect all our oligonucleotide drug products to consist of drug substance formulated in either saline, buffered saline, or some other diluent appropriate for intrathecal, intraocular, subcutaneous, or intravenous injection. These types of formulations can be manufactured using common processes and readily available materials. We are establishing agreements with a variety of contractors that are suitably equipped to manufacture, package, and test these types of oligonucleotide drug product formulations for subsequent shipment to clinical sites. Several of these manufacturers would also be capable of formulation and packaging for commercial use.

Competition

The biotechnology and biopharmaceutical industries, and the genetic medicines fields, are characterized by rapid evolution of technologies, fierce competition and strong defense of intellectual property. Any product candidates that we successfully develop and commercialize will have to compete with existing therapies and new therapies that may become available in the future. While we believe that our technology, development experience and scientific knowledge in the field of biologics, RNA splicing, and antisense oligonucleotide chemistry provide us with competitive advantages, we face potential competition from many different sources, including major pharmaceutical, specialty pharmaceutical and biotechnology companies, academic institutions and governmental agencies, and public and private research institutions that conduct research, seek patent protection, and establish collaborative arrangements for research, development, manufacturing and commercialization.

While therapeutic modalities, including gene therapy, gene editing, modified RNA and protein-based drugs, are currently being developed to address monogenic diseases, most of these approaches are focused on autosomal recessive or autosomal dominant gain-of-function diseases. The nature and fundamental limitations of these approaches make them less suited for addressing the underlying cause of autosomal dominant haploinsufficiencies. Other next generation antisense oligonucleotides have also generally had limited success in upregulating gene expression of haploinsufficiencies, due to a focus on indirectly and weakly validated mechanisms of action such as targeting microRNAs or long non-coding RNAs that are associated with a gene transcript. We are pioneers in developing disease-modifying therapies to treat haploinsufficiencies and are uniquely positioned to exploit this significant opportunity with our TANGO platform.

If our current product candidate, STK-001, is approved for the treatment of Dravet syndrome, it may compete with other products currently marketed or in development. Currently marketed antiepileptic drugs range from cannabidiols, such as GW Pharmaceuticals, plc's Epidiolex, to GABA receptor agonists, such as clobazam, to glutamate blockers, such as topiramate. Encoded Therapeutics, Inc. is also developing a treatment for Dravet syndrome. Many of the currently marketed antiepileptic drugs are available as generics. In addition, numerous compounds are in clinical development for treatment of epilepsy. To our knowledge, the clinical development pipeline includes cannabinoids, 5-HT release stimulants, cholesterol 24-hydroxylase inhibitors, and sodium channel antagonists from a variety of companies. Importantly, we believe none of these small molecule drugs address the underlying genetic cause of Dravet syndrome.

Many of our competitors, either alone or with strategic partners, have substantially greater financial, technical and human resources than we do. Accordingly, our competitors may be more successful than us in research and development, manufacturing, preclinical testing, conducting clinical trials, obtaining approval for treatments and achieving widespread market acceptance, rendering our treatments obsolete or non-competitive. Merger and acquisition activity in the biotechnology and biopharmaceutical industries may result in even more resources being concentrated among a smaller number of our competitors. These companies also compete with us in recruiting and retaining qualified scientific and management personnel, establishing clinical trial sites and patient registration for clinical trials and acquiring technologies complementary to, or necessary for, our programs. Smaller or early-stage companies may also prove to be significant competitors, particularly through collaborative arrangements with large and established companies. Our commercial opportunity could be substantially limited if our competitors develop and commercialize products that are more effective, safer, less toxic, more convenient or less expensive than our comparable products. In geographies that are critical to our commercial success, competitors may also obtain regulatory approvals before us, resulting in our competitors building a strong market position in advance of the entry of our products. In addition, our ability to compete may be affected in many cases by insurers or other third-party payors seeking to encourage the use of other drugs. The key competitive factors affecting the successful of all of our programs are likely to be their efficacy, safety, convenience and availability of reimbursement.

Reimbursement

The regulations that govern pricing and reimbursement for new drugs vary widely from country to country. Some countries require approval of the sale price of a drug before it can be marketed. In many countries, the pricing review period begins after marketing approval is granted. In some foreign markets, prescription biopharmaceutical pricing remains subject to continuing governmental control even after initial approval is granted. As a result, a drug company can obtain regulatory approval for a product in a country, but then be subject to price regulations that delay commercial launch of that product.

A drug company's ability to commercialize any products successfully will also depend in part on the extent to which coverage and adequate reimbursement for these products and related treatments will be available from government authorities, private health insurers and other organizations. Even if one or more products are successfully brought to the market, these products may not be considered cost effective, and the amount reimbursed for such products may be insufficient to allow them to be sold on a competitive basis. Third-party payors who reimburse patients or healthcare providers, such as government plans, are requiring that drug companies provide them with predetermined discounts from list prices and are seeking to reduce the prices charged or the amounts reimbursed for biopharmaceutical products.

Significant delays can occur in obtaining reimbursement for newly-approved drugs or therapeutic biologics, and coverage may be more limited than the purposes for which the drug or therapeutic biologic is approved by the FDA or similar foreign regulatory authorities. Moreover, eligibility for reimbursement does not imply that any drug will be reimbursed in all cases or at a rate that covers a drug company's costs, including research, development, manufacture, sale and distribution.

Interim reimbursement levels for new drugs, if applicable, may also be insufficient to cover a drug company's costs and may not be made permanent. Reimbursement rates may be based on payments allowed for lower cost drugs or therapeutic biologics that are already reimbursed, may be incorporated into existing payments for other services and may reflect budgetary constraints or imperfections in Medicare data. Net prices for drugs or therapeutic biologics may be reduced by mandatory discounts or rebates required by government healthcare programs or private payors and by any future relaxation of laws that presently restrict imports of drugs or therapeutic biologics from countries where they may be sold at lower prices than in the United States. Further, no uniform policy for coverage and reimbursement exists in the United States. Third-party payors often rely upon Medicare coverage policy and payment limitations in setting their own reimbursement rates, but also have their own methods and approval process apart from Medicare determinations. Therefore, coverage and reimbursement can differ significantly from payor to payor.

Intellectual property

We strive to protect and enhance the proprietary technology, inventions and improvements that are commercially important to our business, including obtaining, maintaining and defending patent rights, whether developed internally or licensed from third parties. Our policy is to seek to protect our proprietary position by, among, other methods, pursuing and obtaining patent protection in the United States and in jurisdictions outside of the United States related to our proprietary technology, inventions, improvements, platforms and product candidates that are important to the development and implementation of our business. Our patent portfolio, including in-licensed patents and patent applications, is intended to cover, but is not limited to, our technology platforms, product candidates and components thereof, their methods of use and processes for their manufacture, and any other inventions that are commercially important to our business. We also rely on trade secret protection of our confidential information and know-how relating to our proprietary technology, platforms and product candidates, continuing innovation, and in-licensing opportunities to develop, strengthen, and maintain our position in our TANGO platform and product candidates. Our commercial success may depend

in part on our ability to obtain and maintain patent and other proprietary protection for our technology, inventions and improvements; to preserve the confidentiality of our trade secrets; to maintain our licenses to use intellectual property owned or controlled by third parties; to defend and enforce our proprietary rights, including our patents; to defend against challenges and assertions by third parties of their purported intellectual property rights; and to operate without infringement of valid and enforceable patents and other proprietary rights of third parties.

With respect to our TANGO platform, we have exclusively licensed intellectual property for our TANGO technology from the University of Southampton and Cold Spring Harbor Laboratory, which includes issued U.S. patents and pending U.S. and foreign patent applications that cover the TANGO mechanisms. As of March 31, 2019, there are approximately three issued U.S. patents, approximately two pending U.S. patent applications and approximately 24 pending foreign patent applications that we have licensed from the University of Southampton, which are anticipated to expire between 2035 and 2036, absent any patent term adjustments or extensions. As of March 31, 2019, there is one issued U.S. patent, one pending U.S. patent application and approximately ten pending foreign patent applications that we have licensed from Cold Spring Harbor Laboratory, which are anticipated to expire in 2035, absent any patent term adjustments or extensions.

Separately, we have filed patent applications with claims that are intended to cover compositions of matter of oligonucleotides designed to target specific elements in genes for more than 140 genetic diseases that we believe are amenable to upregulation of target protein expression using our TANGO platform. As of March 31, 2019, these filed patent applications include approximately two PCT international applications, approximately seven such U.S. patent applications, and approximately 34 such foreign patent applications. Any patents that may issue from these currently pending patent applications are expected to expire between 2036 and 2040, absent any patent term adjustments or extensions.

With respect to STK-001, as of March 31, 2019, we have exclusively licensed one issued U.S. patent and one pending U.S. patent application that cover the mechanism of action of STK-001, as well as approximately 13 pending foreign patent applications. The issued patent and any patents that may issue from these pending patent applications are expected to expire between 2035 and 2036, absent any patent term adjustments or extensions. As of March 31, 2019, we also own one pending PCT international application and approximately three pending U.S. patent applications relating to STK-001, and any patents that may issue from these pending patent applications are expected to expire between 2038 and 2040, absent any patent term adjustments or extensions.

The term of individual patents depends upon the laws of the countries in which they are obtained. In most countries in which we file, the patent term is 20 years from the earliest date of filing of a non-provisional patent application. However, the term of United States patents may be extended for delays incurred due to compliance with the FDA requirements or by delays encountered during prosecution that are caused by the United States Patent and Trademark Office, or the USPTO. For example, for drugs that are regulated by the FDA under the Hatch-Waxman Act, it is permitted to extend the term of a patent that covers such drug for up to five years beyond the normal expiration date of the patent. For more information on patent term extensions, see “Business—Government regulation: The Hatch-Waxman Act—Patent term extension”. In the future, if and when our biopharmaceutical product candidates receive FDA approval, we expect to apply for patent term extensions on patents covering those product candidates. We intend to seek patent term extensions to any of our issued patents in any jurisdiction where these are available; however, there is no guarantee that the applicable authorities, including the USPTO and FDA, will agree with our assessment of whether such extensions should be granted, and even if granted, the length of such extensions. Our currently issued patents will likely expire on dates ranging from 2035 to 2036, unless we receive patent term extension or patent term adjustment, or both. If patents are issued on our pending patent applications, the resulting patents are projected to expire on dates ranging from 2036 to 2040, unless we receive patent term extension or patent term adjustment, or both.

However, the actual protection afforded by a patent varies on a product-by-product basis, from country-to-country, and depends upon many factors, including the type of patent, the scope of its coverage, the availability of regulatory-related extensions, the availability of legal remedies in a particular country and the validity and enforceability of the patent.

The patent positions of companies like ours are generally uncertain and involve complex legal and factual questions. No consistent policy regarding the scope of claims allowable in patents in the field of genetic therapy has emerged in the United States. The patent situation outside of the United States is even more uncertain. Changes in the patent laws and rules, either by legislation, judicial decisions, or regulatory interpretation in the United States and other countries may diminish our ability to protect our inventions and enforce our intellectual property rights, and more generally could affect the value of our intellectual property. In particular, our ability to stop third parties from making, using, selling, offering to sell, importing or otherwise commercializing any of our patented inventions, either directly or indirectly, will depend in part on our success in obtaining, defending and enforcing patent claims that cover our technology, inventions, and improvements. With respect to both licensed and company-owned intellectual property, we cannot be sure that patents will be granted with respect to any of our pending patent applications or with respect to any patent applications filed by us in the future, nor can we be sure that any of our existing patents or any patents that may be granted to us in the future will be commercially useful in protecting our platform and product candidates and the methods used to manufacture them. Moreover, our issued patents and those that may issue in the future may not guarantee us the right to practice our technology in relation to the commercialization of our platform's product candidates. The area of patent and other intellectual property rights in biotechnology is an evolving one with many risks and uncertainties, and third parties may have blocking patents that could be used to prevent us from commercializing our TANGO platform and product candidates and practicing our proprietary technology. Our issued patents and those that may issue in the future may be challenged, narrowed, circumvented or invalidated, which could limit our ability to stop competitors from marketing related platforms or product candidates or limit the length of the term of patent protection that we may have for our TANGO platform and product candidates. In addition, the rights granted under any issued patents may not provide us with protection or competitive advantages against competitors with similar technology. Furthermore, our competitors may independently develop similar technologies. For these reasons, we may have competition for our TANGO platform and product candidates. Moreover, because of the extensive time required for development, testing and regulatory review of a potential product, it is possible that before any product candidate can be commercialized, any related patent may expire or remain in force for only a short period following commercialization, thereby reducing any advantage of the patent. For this and other risks related to our proprietary technology, inventions, improvements, platforms and product candidates, please see the section entitled "Risk factors—Risks related to our intellectual property."

We intend to file applications for trademark registrations in connection with our product candidates in various jurisdictions, including the United States. We have filed for trademark protection of the Stoke Therapeutics mark with the United States Patent and Trademark Office and foreign patent and trademark organizations. The Stoke Therapeutics mark was registered by the United States Patent and Trademark Office in 2017, in the European Union in 2016, in Japan in 2016, in China in 2017, in India in 2016, and in Singapore in 2016. We have chosen to not file for trademark protection of the TANGO mark.

We also rely on trade secret protection for our confidential and proprietary information. Although we take steps to protect our confidential and proprietary information as trade secrets, including through contractual means with our employees, consultants, outside scientific collaborators, sponsored researchers and other advisors, third parties may independently develop substantially equivalent proprietary information and techniques or otherwise gain access to our trade secrets or disclose our technology. Thus, we may not be able to meaningfully protect our trade secrets. It is our policy to require our employees, consultants, outside scientific collaborators,

sponsored researchers and other advisors to execute confidentiality agreements under the commencement of employment or consulting relationships with us. These agreements provide that all confidential information concerning our business or financial affairs developed or made known to the individual during the individual's relationship with us is to be kept confidential and not disclosed to third parties except in specific circumstances. In the case of employees, the agreements provide that all inventions conceived by the individual, and which are related to our current or planned business or research and development or made during normal working hours, on our premises or using our equipment or proprietary information, are our exclusive property. In many cases our confidentiality and other agreements with consultants, outside scientific collaborators, sponsored researchers and other advisors require them to assign or grant us licenses to inventions they invent as a result of the work or services they render under such agreements or grant us an option to negotiate a license to use such inventions. Despite these efforts, we cannot provide any assurances that all such agreements have been duly executed, and any of these parties may breach the agreements and disclose our proprietary information, and we may not be able to obtain adequate remedies for such breaches.

We also seek to preserve the integrity and confidentiality of our proprietary technology and processes by maintaining physical security of our premises and physical and electronic security of our information technology systems. Although we have confidence in these individuals, organizations and systems, agreements or security measures may be breached, and we may not have adequate remedies for any breach. To the extent that our employees, contractors, consultants, collaborators and advisors use intellectual property owned by others in their work for us, disputes may arise as to the rights in relation to the resulting know-how or inventions. For more information, please see the section entitled "Risk factors – Risks related to our intellectual property."

License agreements

Cold Spring Harbor Laboratory

In July 2015, we entered into a worldwide license agreement with CSHL, or the CSHL Agreement, with respect to the TANGO patents. Under the CSHL Agreement, we receive an exclusive (except with respect to certain government rights and non-exclusive licenses), worldwide license under certain patents and applications relating to TANGO. As part of the CSHL Agreement, we granted CSHL 164,927 shares of common stock. The CSHL Agreement obligates us to make additional payments that are contingent upon certain milestones being achieved as well as royalties in the low- to mid-single digits on future product sales. These royalty obligations last on a product-by-product and country-by-country basis until the latest of (i) the expiration of the last valid claim of a patent covering a subject product or (ii) the expiration of any regulatory exclusivity for the subject product in a country. In addition, if we sublicense rights under the CSHL Agreement, we are required to pay a low double-digit percentage of the sublicense revenue to CSHL, which may be reduced upon achievement of certain milestones for the applicable subject product. The maximum aggregate potential milestone payments payable total approximately $900,000. Additionally, certain licenses under the CSHL Agreement require us to reimburse CSHL for certain past and ongoing patent related expenses, however there were no expenses related to these reimbursable patent costs during the years ended December 31, 2018 and 2017 or for the three months ended March 31, 2019 and 2018.

University of Southampton

In April 2016, we entered into an exclusive, worldwide license agreement with the University of Southampton, or the Southampton Agreement, whereby we acquired rights to foundational technologies related to our TANGO technology. Under the Southampton Agreement, we receive an exclusive, worldwide license under certain licensed patents and applications relating to TANGO. As part of the Southampton Agreement, we paid 55,000 pounds sterling (approximately $72,000 as of the date thereof) as an up-front license fee. Under the Southampton Agreement, we may be obligated to make additional payments that are contingent upon certain milestones being achieved, as well as royalties in the low- to mid-single digits on future product sales. These

royalty obligations survive until the latest of (i) the expiration of the last valid claim of a licensed patent covering a subject product or (ii) the expiration of any regulatory exclusivity for the subject product in a country. In addition, if we sublicense our rights under the Southampton Agreement, we are required to pay a mid-single digit percentage of the sublicense revenue to the University of Southampton. The maximum aggregate potential milestone payments payable by us total approximately 400,000 pounds sterling (approximately $518,000 as of March 31, 2019). As of March 31, 2019, we have recorded no liabilities under the Southampton Agreement.

Government regulation

FDA approval process

In the United States, pharmaceutical products are subject to extensive regulation by FDA. The Federal Food, Drug, and Cosmetic Act and other federal and state statutes and regulations govern, among other things, the research, development, testing, manufacture, storage, recordkeeping, approval, labeling, promotion and marketing, distribution, post-approval monitoring and reporting, sampling and import and export of pharmaceutical products. Failure to comply with applicable U.S. requirements may subject a company to a variety of administrative or judicial sanctions, such as FDA refusal to approve pending new drug applications, or NDAs, warning or untitled letters, product recalls, product seizures, total or partial suspension of production or distribution, injunctions, fines, civil penalties and criminal prosecution.

Pharmaceutical product development for a new product or certain changes to an approved product in the U.S. typically involves preclinical laboratory and animal tests, the submission to FDA of an IND which must become effective before clinical testing may commence, and adequate and well-controlled clinical trials to establish the safety and effectiveness of the drug for each indication for which FDA approval is sought. Satisfaction of FDA pre-market approval requirements typically takes many years and the actual time required may vary substantially based upon the type, complexity and novelty of the product or disease.

Preclinical tests include laboratory evaluation of product chemistry, formulation and toxicity, as well as animal trials to assess the characteristics and potential safety and efficacy of the product. The conduct of the preclinical tests must comply with federal regulations and requirements, including good laboratory practices. The results of preclinical testing are submitted to FDA as part of an IND along with other information, including information about product chemistry, manufacturing and controls, and a proposed clinical trial protocol. Long-term preclinical tests, such as animal tests of reproductive toxicity and carcinogenicity, may continue after the IND is submitted.

A 30-day waiting period after the submission of each IND is required prior to the commencement of clinical testing in humans. If FDA has neither commented on nor questioned the IND within this 30-day period, the clinical trial proposed in the IND may begin.

Clinical trials involve the administration of the investigational new drug to healthy volunteers or patients under the supervision of a qualified investigator. Clinical trials must be conducted: (i) in compliance with federal regulations; (ii) in compliance with good clinical practice, or GCP, an international standard meant to protect the rights and health of patients and to define the roles of clinical trial sponsors, administrators and monitors; as well as (iii) under protocols detailing the objectives of the trial, the parameters to be used in monitoring safety and the effectiveness criteria to be evaluated. Each protocol involving testing on U.S. patients and subsequent protocol amendments must be submitted to FDA as part of the IND.

FDA may order the temporary, or permanent, discontinuation of a clinical trial at any time, or impose other sanctions, if it believes that the clinical trial either is not being conducted in accordance with FDA requirements or presents an unacceptable risk to the clinical trial patients. The study protocol and informed consent information for patients in clinical trials must also be submitted to an institutional review board, or IRB, for

approval. An IRB may also require the clinical trial at the site to be halted, either temporarily or permanently, for failure to comply with the IRB's requirements, or may impose other conditions.

Clinical trials to support NDAs for marketing approval are typically conducted in three sequential phases, but the phases may overlap. In Phase 1, the initial introduction of the drug into healthy human subjects or patients, the drug is tested to assess metabolism, pharmacokinetics, pharmacological actions, side effects associated with increasing doses, and, if possible, early evidence of effectiveness. Phase 2 usually involves trials in a limited patient population to determine the effectiveness of the drug for a particular indication, dosage tolerance and optimum dosage, and to identify common adverse effects and safety risks. If a compound demonstrates evidence of effectiveness and an acceptable safety profile in Phase 2 evaluations, Phase 3 trials are undertaken to obtain the additional information about clinical efficacy and safety in a larger number of patients, typically at geographically dispersed clinical trial sites, to permit FDA to evaluate the overall benefit-risk relationship of the drug and to provide adequate information for the labeling of the drug. In most cases FDA requires two adequate and well-controlled Phase 3 clinical trials to demonstrate the efficacy of the drug. A single Phase 3 trial with other confirmatory evidence may be sufficient in rare instances, such as where the study is a large multicenter trial demonstrating internal consistency and a statistically very persuasive finding of a clinically meaningful effect on mortality, irreversible morbidity or prevention of a disease with a potentially serious outcome and confirmation of the result in a second trial would be practically or ethically impossible.

After completion of the required clinical testing, an NDA is prepared and submitted to FDA. FDA approval of the NDA is required before marketing of the product may begin in the U.S. The NDA must include the results of all preclinical, clinical and other testing and a compilation of data relating to the product's pharmacology, chemistry, manufacture and controls. The cost of preparing and submitting an NDA is substantial. The submission of most NDAs is additionally subject to a substantial application user fee, currently exceeding $2,580,000 for fiscal year 2019, and the manufacturer and sponsor under an approved NDA are also subject to annual program fees, currently exceeding $300,000 for each prescription product. These fees are typically increased annually. Sponsors of applications for drugs granted Orphan Drug Designation are exempt from these user fees.

FDA has 60 days from its receipt of an NDA to determine whether the application will be accepted for filing based on the agency's threshold determination that it is sufficiently complete to permit substantive review. Once the submission is accepted for filing, FDA begins an in-depth review. FDA has agreed to certain performance goals in the review of new drug applications to encourage timeliness. Most applications for standard review drug products are reviewed within ten to twelve months; most applications for priority review drugs are reviewed in six to eight months. Priority review can be applied to drugs that FDA determines offer major advances in treatment or provide a treatment where no adequate therapy exists. The review process for both standard and priority review may be extended by FDA for three additional months to consider certain late-submitted information, or information intended to clarify information already provided in the submission.

FDA may also refer applications for novel drug products, or drug products that present difficult questions of safety or efficacy, to an outside advisory committee—typically a panel that includes clinicians and other experts—for review, evaluation and a recommendation as to whether the application should be approved. FDA is not bound by the recommendation of an advisory committee, but it generally follows such recommendations.

Before approving an NDA, FDA will typically inspect one or more clinical sites to assure compliance with GCP. Additionally, FDA will inspect the facility or the facilities at which the drug is manufactured. FDA will not approve the product unless compliance with current good manufacturing practices, or cGMPs, is satisfactory and the NDA contains data that provide substantial evidence that the drug is safe and effective in the indication studied.

After FDA evaluates the NDA and the manufacturing facilities, it issues either an approval letter or a complete response letter. A complete response letter generally outlines the deficiencies in the submission and may

require substantial additional testing, or information, in order for FDA to reconsider the application. If, or when, those deficiencies have been addressed to FDA's satisfaction in a resubmission of the NDA, FDA will issue an approval letter. FDA has committed to reviewing such resubmissions in two or six months depending on the type of information included.

An approval letter authorizes commercial marketing of the drug with specific prescribing information for specific indications. As a condition of NDA approval, FDA may require a risk evaluation and mitigation strategy, or REMS, to help ensure that the benefits of the drug outweigh the potential risks. REMS can include medication guides, communication plans for healthcare professionals, and elements to assure safe use, or ETASU. ETASU can include, but are not limited to, special training or certification for prescribing or dispensing, dispensing only under certain circumstances, special monitoring and the use of patient registries. The requirement for a REMS can materially affect the potential market and profitability of the drug. Moreover, product approval may require substantial post-approval testing and surveillance to monitor the drug's safety or efficacy. Once granted, product approvals may be withdrawn if compliance with regulatory standards is not maintained or problems are identified following initial marketing.

Changes to some of the conditions established in an approved application, including changes in indications, labeling, or manufacturing processes or facilities, require submission and FDA approval of a new NDA or NDA supplement before the change can be implemented. An NDA supplement for a new indication typically requires clinical data similar to that in the original application, and FDA uses the same procedures and actions in reviewing NDA supplements as it does in reviewing NDAs.

Fast Track Designation and accelerated approval

FDA is required to facilitate the development, and expedite the review, of drugs that are intended for the treatment of a serious or life-threatening disease or condition for which there is no effective treatment and which demonstrate the potential to address unmet medical needs for the condition. Under the Fast Track program, the sponsor of a new drug candidate may request that FDA designate the drug candidate for a specific indication as a Fast Track drug concurrent with, or after, the filing of the IND for the drug candidate. FDA must determine if the drug candidate qualifies for Fast Track Designation within 60 days of receipt of the sponsor's request.

Under the Fast Track program and FDA's accelerated approval regulations, FDA may approve a drug for a serious or life-threatening illness that provides meaningful therapeutic benefit to patients over existing treatments based upon a surrogate endpoint that is reasonably likely to predict clinical benefit, or on a clinical endpoint that can be measured earlier than irreversible morbidity or mortality, that is reasonably likely to predict an effect on irreversible morbidity or mortality or other clinical benefit, taking into account the severity, rarity, or prevalence of the condition and the availability or lack of alternative treatments.

In clinical trials, a surrogate endpoint is a measurement of laboratory or clinical signs of a disease or condition that substitutes for a direct measurement of how a patient feels, functions, or survives. Surrogate endpoints can often be measured more easily or more rapidly than clinical endpoints. A drug candidate approved on this basis is subject to rigorous post-marketing compliance requirements, including the completion of Phase 4 or post-approval clinical trials to confirm the effect on the clinical endpoint. Failure to conduct required post-approval studies, or confirm a clinical benefit during post-marketing studies, will allow FDA to withdraw the drug from the market on an expedited basis. All promotional materials for drug candidates approved under accelerated regulations are subject to priority review by FDA.

If a submission is granted Fast Track Designation, the sponsor may engage in more frequent interactions with FDA, and FDA may review sections of the NDA before the application is complete. This rolling review is available if the applicant provides, and FDA approves, a schedule for the submission of the remaining information and

the applicant pays applicable user fees. However, FDA's time period goal for reviewing an application does not begin until the last section of the NDA is submitted. Additionally, Fast Track Designation may be withdrawn by FDA if FDA believes that the designation is no longer supported by data emerging in the clinical trial process.

Breakthrough Therapy Designation

FDA is also required to expedite the development and review of the application for approval of drugs that are intended to treat a serious or life-threatening disease or condition where preliminary clinical evidence indicates that the drug may demonstrate substantial improvement over existing therapies on one or more clinically significant endpoints. Under the Breakthrough Therapy program, the sponsor of a new drug candidate may request that FDA designate the drug candidate for a specific indication as a breakthrough therapy concurrent with, or after, the filing of the IND for the drug candidate. FDA must determine if the drug candidate qualifies for Breakthrough Therapy designation within 60 days of receipt of the sponsor's request.

Orphan Drugs

Under the Orphan Drug Act, FDA may grant Orphan Drug Designation to drugs intended to treat a rare disease or condition—generally a disease or condition that affects fewer than 200,000 individuals in the U.S. Orphan Drug designation must be requested before submitting an NDA. After FDA grants Orphan Drug Designation, the generic identity of the drug and its potential orphan use are disclosed publicly by FDA. Orphan Drug Designation does not convey any advantage in, or shorten the duration of, the regulatory review and approval process. The first NDA applicant to receive FDA approval for a particular active ingredient to treat a particular disease with FDA Orphan Drug Designation is entitled to a seven-year exclusive marketing period in the U.S. for that product, for that indication. During the seven-year exclusivity period, FDA may not approve any other applications to market the same drug for the same disease, except in limited circumstances, such as a showing of clinical superiority to the product with orphan drug exclusivity. Orphan drug exclusivity does not prevent FDA from approving a different drug for the same disease or condition, or the same drug for a different disease or condition. Among the other benefits of Orphan Drug Designation are tax credits for certain research and an exemption from the NDA application user fee.

Rare Pediatric Disease Priority Review Voucher Program

Under the Rare Pediatric Disease Priority Review Voucher program, FDA may award a priority review voucher to the sponsor of an approved marketing application for a product that treats or prevents a rare pediatric disease. The voucher entitles the sponsor to priority review of one subsequent marketing application.

A voucher may be awarded only for an approved rare pediatric disease product application. A rare pediatric disease product application is an NDA for a product that treats or prevents a serious or life-threatening disease in which the serious or life-threatening manifestations primarily affect individuals aged from birth to 18 years; in general, the disease must affect fewer than 200,000 such individuals in the U.S.; the NDA must be deemed eligible for priority review; the NDA must not seek approval for a different adult indication (i.e., for a different disease/condition); the product must not contain an active ingredient that has been previously approved by FDA; and the NDA must rely on clinical data derived from studies examining a pediatric population such that the approved product can be adequately labeled for the pediatric population. Before NDA approval, FDA may designate a product in development as a product for a rare pediatric disease, but such designation is not required to receive a voucher.

To receive a rare pediatric disease priority review voucher, a sponsor must notify FDA, upon submission of the NDA, of its intent to request a voucher. If FDA determines that the NDA is a rare pediatric disease product application, and if the NDA is approved, FDA will award the sponsor of the NDA a voucher upon approval of the NDA. FDA may revoke a rare pediatric disease priority review voucher if the product for which it was awarded is not marketed in the U.S. within 365 days of the product's approval.

The voucher, which is transferable to another sponsor, may be submitted with a subsequent NDA or biologics license application, or BLA, and entitles the holder to priority review of the accompanying NDA or BLA. The sponsor submitting the priority review voucher must notify FDA of is intent to submit the voucher with the NDA or BLA at least 90 days prior to submission of the NDA or BLA and must pay a priority review user fee in addition to any other required user fee ($2,457,140 in fiscal year 2019). FDA must take action on an NDA or BLA under priority review within six months of receipt of the NDA or BLA.

The Rare Pediatric Disease Priority Review Voucher program was reauthorized in the 21st Century Cures Act, allowing a product that is designated as a product for a rare pediatric disease prior to October 1, 2020 to be eligible to receive a rare pediatric disease priority review voucher upon approval of a qualifying application prior to October 1, 2022.

Post-approval requirements

Once an NDA is approved, a product will be subject to certain post-approval requirements. For instance, FDA closely regulates the post-approval marketing and promotion of drugs, including standards and regulations for direct-to-consumer advertising, off-label promotion, industry-sponsored scientific and educational activities and promotional activities involving the internet. Drugs may be marketed only for the approved indications and in accordance with the provisions of the approved labeling.

Adverse event reporting and submission of periodic reports are required following FDA approval of an NDA. FDA also may require post-marketing testing, known as Phase 4 testing, risk evaluation and mitigation strategies, or REMS, and surveillance to monitor the effects of an approved product, or FDA may place conditions on an approval that could restrict the distribution or use of the product. In addition, quality control, drug manufacture, packaging and labeling procedures must continue to conform to cGMPs after approval. Drug manufacturers and certain of their subcontractors are required to register their establishments with FDA and certain state agencies. Registration with FDA subjects entities to periodic unannounced inspections by FDA, during which the Agency inspects manufacturing facilities to assess compliance with cGMPs. Accordingly, manufacturers must continue to expend time, money and effort in the areas of production and quality-control to maintain compliance with cGMPs. Regulatory authorities may withdraw product approvals or request product recalls if a company fails to comply with regulatory standards, if it encounters problems following initial marketing, or if previously unrecognized problems are subsequently discovered.

Pediatric information

Under the Pediatric Research Equity Act, or PREA, NDAs or supplements to NDAs must contain data to assess the safety and effectiveness of the drug for the claimed indications in all relevant pediatric subpopulations and to support dosing and administration for each pediatric subpopulation for which the drug is safe and effective. FDA may grant full or partial waivers, or deferrals, for submission of data. With certain exceptions, PREA does not apply to any drug for an indication for which orphan designation has been granted.

The Best Pharmaceuticals for Children Act, or BPCA, provides NDA holders a six-month extension of any exclusivity—patent or nonpatent—for a drug if certain conditions are met. Conditions for exclusivity include FDA's determination that information relating to the use of a new drug in the pediatric population may produce health benefits in that population, FDA making a written request for pediatric studies, and the applicant agreeing to perform, and reporting on, the requested studies within the statutory timeframe. Applications under the BPCA are treated as priority applications, with all of the benefits that designation confers.

Disclosure of clinical trial information

Sponsors of clinical trials of FDA regulated products, including drugs, are required to register and disclose certain clinical trial information. Information related to the product, patient population, phase of investigation,

study sites and investigators, and other aspects of the clinical trial is then made public as part of the registration. Sponsors are also obligated to discuss the results of their clinical trials after completion. Disclosure of the results of these trials can be delayed in certain circumstances for up to two years after the date of completion of the trial. Competitors may use this publicly available information to gain knowledge regarding the progress of development programs.

The Hatch-Waxman Act

Orange Book listing

In seeking approval for a drug through an NDA, applicants are required to list with the FDA each patent whose claims cover the applicant's product. Upon approval of a drug, each of the patents listed in the application for the drug is then published in the FDA's Approved Drug Products with Therapeutic Equivalence Evaluations, commonly known as the Orange Book. Drugs listed in the Orange Book can, in turn, be cited by potential generic competitors in support of approval of an abbreviated new drug application, or ANDA. An ANDA provides for marketing of a drug product that has the same active ingredients in the same strengths and dosage form as the listed drug and has been shown through bioequivalence testing to be therapeutically equivalent to the listed drug. Other than the requirement for bioequivalence testing, ANDA applicants are not required to conduct, or submit results of, preclinical or clinical tests to prove the safety or effectiveness of their drug product. Drugs approved in this way are commonly referred to as "generic equivalents" to the listed drug and can often be substituted by pharmacists under prescriptions written for the original listed drug.

The ANDA applicant is required to certify to the FDA concerning any patents listed for the approved product in the FDA's Orange Book. Specifically, the applicant must certify that (i) the required patent information has not been filed; (ii) the listed patent has expired; (iii) the listed patent has not expired but will expire on a particular date and approval is sought after patent expiration; or (iv) the listed patent is invalid or will not be infringed by the new product. The ANDA applicant may also elect to submit a section viii statement certifying that its proposed ANDA label does not contain (or carve out) any language regarding the patented method-of-use rather than certify to a listed method-of-use patent. If the applicant does not challenge the listed patents, the ANDA application will not be approved until all the listed patents claiming the referenced product have expired. A certification that the new product will not infringe the already approved product's listed patents, or that such patents are invalid, is called a Paragraph IV certification. If the ANDA applicant has provided a Paragraph IV certification to the FDA, the applicant must also send notice of the Paragraph IV certification to the NDA and patent holders once the ANDA has been accepted for filing by the FDA. The NDA and patent holders may then initiate a patent infringement lawsuit in response to the notice of the Paragraph IV certification. The filing of a patent infringement lawsuit within 45 days of the receipt of a Paragraph IV certification automatically prevents the FDA from approving the ANDA until the earlier of 30 months, expiration of the patent, settlement of the lawsuit, or a decision in the infringement case that is favorable to the ANDA applicant.

The ANDA application also will not be approved until any applicable non-patent exclusivity listed in the Orange Book for the referenced product has expired.

Exclusivity

Upon NDA approval of a new chemical entity, or NCE, which is a drug that contains no active moiety that has been approved by FDA in any other NDA, that drug receives five years of marketing exclusivity during which FDA cannot receive any ANDA seeking approval of a generic version of that drug. Certain changes to a drug, such as the addition of a new indication to the package insert, are associated with a three-year period of exclusivity during which FDA cannot approval an ANDA for a generic drug that includes the change. An ANDA may be submitted one year before NCE exclusivity expires if a Paragraph IV certification is filed. If there is no

listed patent in the Orange Book, there may not be a Paragraph IV certification, and, thus, no ANDA may be filed before the expiration of the exclusivity period.

Patent term extension

After NDA approval, owners of relevant drug patents may apply for up to a five-year patent extension. The allowable patent term extension is calculated as half of the drug's testing phase (the time between IND application and NDA submission) and all of the review phase (the time between NDA submission and approval up to a maximum of five years). The time can be shortened if FDA determines that the applicant did not pursue approval with due diligence. The total patent term after the extension may not exceed 14 years from the date of product approval. Only one patent applicable to an approved drug is eligible for extension and only those claims covering the approved drug, a method for using it, or a method for manufacturing it may be extended and the application for the extension must be submitted prior to the expiration of the patent. For patents that might expire during the application phase, the patent owner may request an interim patent extension. An interim patent extension increases the patent term by one year and may be renewed up to four times. For each interim patent extension granted, the post-approval patent extension is reduced by one year. The director of the United States Patent and Trademark Office must determine that approval of the drug covered by the patent for which a patent extension is being sought is likely. Interim patent extensions are not available for a drug for which an NDA has not been submitted.

Foreign regulation

In addition to regulations in the United States, we will be subject to a variety of foreign regulations governing clinical trials and commercial sales and distribution of our product candidates to the extent we choose to sell any products outside of the United States. Whether or not we obtain FDA approval for a product, we must obtain approval of a product by regulatory authorities of foreign countries before we can commence clinical trials or marketing of the product in those countries. The approval process varies from country to country and the time may be longer or shorter than that required for FDA approval. The requirements governing the conduct of clinical trials, product licensing, pricing and reimbursement vary greatly from country to country. As in the United States, post-approval regulatory requirements, such as those regarding product manufacture, marketing, or distribution would apply to any product that is approved outside the United States.

Other healthcare laws

In addition to FDA restrictions on marketing of pharmaceutical products, several other types of state and federal laws have been applied to restrict certain general business and marketing practices in the pharmaceutical industry in recent years. These laws include anti-kickback statutes, false claims statutes and other healthcare laws and regulations.

The federal Anti-Kickback Statute prohibits, among other things, knowingly and willfully offering, paying, soliciting or receiving remuneration to induce, or in return for, purchasing, leasing, ordering or arranging for the purchase, lease or order of any healthcare item or service reimbursable under Medicare, Medicaid, or other federally financed healthcare programs. The Patient Protection and Affordable Care Act as amended by the Health Care and Education Reconciliation Act, collectively, the ACA, amended the intent element of the federal statute so that a person or entity no longer needs to have actual knowledge of the statute or specific intent to violate it in order to commit a violation. This statute has been interpreted to apply to arrangements between pharmaceutical manufacturers on the one hand and prescribers, purchasers and formulary managers on the other. Although there are a number of statutory exceptions and regulatory safe harbors protecting certain common activities from prosecution or other regulatory sanctions, the exceptions and safe harbors are drawn narrowly, and practices that involve remuneration intended to induce prescribing, purchases or recommendations may be subject to scrutiny if they do not qualify for an exception or safe harbor.

Federal civil and criminal false claims laws, including the federal civil False Claims Act, prohibit any person or entity from knowingly presenting, or causing to be presented, a false claim for payment to the federal government, or knowingly making, or causing to be made, a false statement to have a false claim paid. This includes claims made to programs where the federal government reimburses, such as Medicaid, as well as programs where the federal government is a direct purchaser, such as when it purchases off the Federal Supply Schedule. Recently, several pharmaceutical and other healthcare companies have been prosecuted under these laws for allegedly inflating drug prices they report to pricing services, which in turn were used by the government to set Medicare and Medicaid reimbursement rates, and for allegedly providing free product to customers with the expectation that the customers would bill federal programs for the product. In addition, certain marketing practices, including off-label promotion, may also violate false claims laws. Additionally, the ACA amended the federal Anti-Kickback Statute such that a violation of that statute can serve as a basis for liability under the federal False Claims Act. Most states also have statutes or regulations similar to the federal Anti-Kickback Statute and False Claims Act, which apply to items and services reimbursed under Medicaid and other state programs, or, in several states, apply regardless of the payor.

Other federal statutes pertaining to healthcare fraud and abuse include the civil monetary penalties statute, which prohibits, among other things, the offer or payment of remuneration to a Medicaid or Medicare beneficiary that the offerer or payor knows or should know is likely to influence the beneficiary to order a receive a reimbursable item or service from a particular supplier, and the additional federal criminal statutes created by the Health Insurance Portability and Accountability Act of 1996, or HIPAA, which prohibits, among other things, knowingly and willfully executing or attempting to execute a scheme to defraud any healthcare benefit program or obtain by means of false or fraudulent pretenses, representations or promises any money or property owned by or under the control of any healthcare benefit program in connection with the delivery of or payment for healthcare benefits, items or services.

In addition, HIPAA, as amended by the Health Information Technology for Economic and Clinical Health Act of 2009, or HITECH, and their respective implementing regulations, including the Final Omnibus Rule published on January 25, 2013, impose obligations on certain healthcare providers, health plans, and healthcare clearinghouses, known as covered entities, as well as their business associates that perform certain services involving the storage, use or disclosure of individually identifiable health information, including mandatory contractual terms, with respect to safeguarding the privacy, security, and transmission of individually identifiable health information, and require notification to affected individuals and regulatory authorities of certain breaches of security of individually identifiable health information. HITECH increased the civil and criminal penalties that may be imposed against covered entities, business associates and possibly other persons, and gave state attorneys general new authority to file civil actions for damages or injunctions in federal courts to enforce the federal HIPAA laws and seek attorney's fees and costs associated with pursuing federal civil actions. In addition, many state laws govern the privacy and security of health information in certain circumstances, many of which differ from each other in significant ways and may not have the same effect.

Further, pursuant to the ACA, the Centers for Medicare & Medicaid Services, or CMS, has issued a final rule that requires manufacturers of prescription drugs to collect and report information on certain payments or transfers of value to physicians and teaching hospitals, as well as investment interests held by physicians and their immediate family members. The first reports were due in 2014 and must be submitted on an annual basis. The reported data is made available in searchable form on a public website on an annual basis. Failure to submit required information may result in civil monetary penalties. Effective January 1, 2022, reporting on transfers of value to physician assistants, nurse practitioners or clinical nurse specialists, certified registered nurse anesthetists, and certified nurse-midwives will also be required.

In addition, several states now require prescription drug companies to report certain expenses relating to the marketing and promotion of drug products and to report gifts and payments to individual healthcare

practitioners in these states. Other states prohibit various marketing-related activities, such as the provision of certain kinds of gifts or meals. Still other states require the posting of information relating to clinical studies and their outcomes. Some states require the reporting of certain pricing information, including information pertaining to and justifying price increases, or prohibit prescription drug price gouging. In addition, states such as California, Connecticut, Nevada, and Massachusetts require pharmaceutical companies to implement compliance programs and/or marketing codes. Several additional states are considering similar proposals. Certain states and local jurisdictions also require the registration of pharmaceutical sales representatives. Compliance with these laws is difficult and time consuming, and companies that do not comply with these state laws face civil penalties.

Efforts to ensure that business arrangements with third parties comply with applicable healthcare laws and regulations involve substantial costs. If a drug company's operations are found to be in violation of any such requirements, it may be subject to significant penalties, including civil, criminal and administrative penalties, damages, fines, disgorgement, imprisonment, the curtailment or restructuring of its operations, loss of eligibility to obtain approvals from the FDA, exclusion from participation in government contracting, healthcare reimbursement or other federal or state government healthcare programs, including Medicare and Medicaid, integrity oversight and reporting obligations, imprisonment, and reputational harm. Although effective compliance programs can mitigate the risk of investigation and prosecution for violations of these laws, these risks cannot be entirely eliminated. Any action for an alleged or suspected violation can cause a drug company to incur significant legal expenses and divert management's attention from the operation of the business, even if such action is successfully defended.

U.S. healthcare reform

In the United States there have been, and continue to be, proposals by the federal government, state governments, regulators and third-party payors to control or manage the increased costs of health care and, more generally, to reform the U.S. healthcare system. The pharmaceutical industry has been a particular focus of these efforts and has been significantly affected by major legislative initiatives For example, in March 2010, the ACA was enacted, which intended to broaden access to health insurance, reduce or constrain the growth of healthcare spending, enhance remedies against fraud and abuse, add new transparency requirements for the healthcare and health insurance industries, impose new taxes and fees on the health industry and impose additional health policy reforms, substantially changed the way healthcare is financed by both governmental and private insurers, and significantly impacts the U.S. pharmaceutical industry. The ACA, among other things, (i) subjected therapeutic biologics to potential competition by lower-cost biosimilars by creating a licensure framework for follow-on biologic products, (ii) proscribed a new methodology by which rebates owed by manufacturers under the Medicaid Drug Rebate Program are calculated for drugs and therapeutic biologics that are inhaled, infused, instilled, implanted or injected, (iii) increased the minimum Medicaid rebates owed by manufacturers under the Medicaid Drug Rebate Program and extended the rebate program to individuals enrolled in Medicaid managed care organizations, (iv) established annual nondeductible fees and taxes on manufacturers of certain branded prescription drugs and therapeutic biologics, apportioned among these entities according to their market share in certain government healthcare programs (v) established a new Medicare Part D coverage gap discount program, in which manufacturers must agree to offer what are now 70% point of-sale discounts off negotiated prices of applicable brand drugs and therapeutic biologics to eligible beneficiaries during their coverage gap period, as a condition for the manufacturer's outpatient drugs and therapeutic biologics to be covered under Medicare Part D, (vi) expanded eligibility criteria for Medicaid programs by, among other things, allowing states to offer Medicaid coverage to additional individuals and by adding new mandatory eligibility categories for individuals with income at or below 133% of the federal poverty level, thereby potentially increasing manufacturers' Medicaid rebate liability, (vii) expanded the entities eligible for discounts under the Public Health program (viii) created a new Patient-Centered Outcomes Research

Institute to oversee, identify priorities in, and conduct comparative clinical effectiveness research, along with funding for such research, and (ix) established a Center for Medicare Innovation at CMS to test innovative payment and service delivery models to lower Medicare and Medicaid spending, potentially including prescription drug spending.

The current U.S. presidential administration and Congress have, and we expect they will continue to, seek to modify, repeal, or otherwise invalidate all, or certain provisions of, the ACA. Since January 2017, the current U.S. presidential administration has issued two executive orders and other directives designed to delay the implementation of certain provisions of the ACA or otherwise circumvent some of the requirements for health insurance mandated by the ACA. For example, on October 12, 2017, the current U.S. presidential administration issued an executive order that expands the use of association health plans and allows anyone to purchase short-term health plans that provide temporary, limited insurance. This executive order also calls for the halt of federal payments to health insurers for cost-sharing reductions previously available to lower-income Americans to afford coverage. There is still uncertainty with respect to the impact this executive order could have on coverage and reimbursement for healthcare items and services covered by plans that were authorized by the ACA. Concurrently, Congress has considered legislation that would repeal or repeal and replace all or part of the ACA. While Congress has not passed comprehensive repeal legislation, two bills affecting the implementation of certain taxes under the ACA have been signed into law. The TCJA, among other things, included a provision repealing, effective January 1, 2019, the tax-based shared responsibility payment imposed by the ACA on certain individuals who fail to maintain qualifying health coverage for all or part of a year that is commonly referred to as the “individual mandate”. Additionally, on January 22, 2018, the current U.S. presidential administration signed a continuing resolution on appropriations for fiscal year 2018 that delayed the implementation of certain ACA-mandated fees, including the so-called “Cadillac” tax on certain high cost employer-sponsored insurance plans, the annual fee imposed on certain health insurance providers based on market share, and the medical device excise tax on non-exempt medical devices. Further, the Bipartisan Budget Act of 2018, or the BBA, among other things, amended the ACA, effective January 1, 2019, to increase from 50% to 70% the point-of-sale discount that is owed by pharmaceutical manufacturers who participate in Medicare Part D and to close the coverage gap in most Medicare drug plans, commonly referred to as the “donut hole”. More recently, in July 2018, CMS published a final rule permitting further collections and payments to and from certain ACA qualified health plans and health insurance issuers under the ACA risk adjustment program in response to the outcome of federal district court litigation regarding the method CMS uses to determine this risk adjustment. There is still uncertainty with respect to the impact the current U.S. presidential administration and the Congress may have, if any, and any changes will likely take time to unfold, and could have an impact on coverage and reimbursement for healthcare items and services covered by plans that were authorized by the ACA. However, we cannot predict the ultimate content, timing or effect of any healthcare reform legislation or the impact of potential legislation on us.

In addition, other legislative changes have been proposed and adopted in the United States since the ACA was enacted to reduce healthcare expenditures. United States federal government agencies also currently face potentially significant spending reductions, which may further impact healthcare expenditures. On August 2, 2011, the Budget Control Act of 2011 among other things, created measures for spending reductions by Congress. A joint select committee on deficit reduction, tasked with recommending a targeted deficit reduction of at least $1.2 trillion for the years 2013 through 2021, was unable to reach required goals, thereby triggering the legislation’s automatic reduction to several government programs. This includes aggregate reductions of Medicare payments to providers of 2% per fiscal year. These reductions went into effect on April 1, 2013 and, due to subsequent legislative amendments to the statute, including the BBA, will remain in effect through 2027 unless additional Congressional action is taken. Moreover, on January 2, 2013, the American Taxpayer Relief Act of 2012 was signed into law, which, among other things, further reduced Medicare payments to several types of providers, including hospitals, imaging centers and cancer treatment centers, and increased the statute of

limitations period for the government to recover overpayments to providers from three to five years. If federal spending is further reduced, anticipated budgetary shortfalls may also impact the ability of relevant agencies, such as the FDA or the National Institutes of Health to continue to function at current levels. Amounts allocated to federal grants and contracts may be reduced or eliminated. These reductions may also impact the ability of relevant agencies to timely review and approve research and development, manufacturing, and marketing activities, which may delay our ability to develop, market and sell any products we may develop.

Moreover, payment methodologies may be subject to changes in healthcare legislation and regulatory initiatives. For example, the Medicare Prescription Drug, Improvement, and Modernization Act of 2003, or MMA, changed the way Medicare covers and pays for pharmaceutical products. The legislation expanded Medicare coverage for drug purchases by the elderly and introduced a new reimbursement methodology based on average sales prices for physician-administered drugs. In addition, this legislation provided authority for limiting the number of drugs that will be covered in any therapeutic class. While the MMA only applies to drug benefits for Medicare beneficiaries, private payors often follow Medicare coverage policy and payment limitations in setting their own reimbursement rates. Therefore, any reduction in reimbursement that results from the MMA may result in a similar reduction in payments from private payors.

Recently there has been heightened governmental scrutiny over the manner in which manufacturers set prices for their marketed products, which has resulted in several Congressional inquiries and proposed and enacted federal and state legislation designed to, among other things, bring more transparency to product pricing, review the relationship between pricing and manufacturer patient programs, and reform government program reimbursement methodologies for drug products. At the federal level, the current U.S. presidential administration's budget proposal for fiscal year 2019 contains further drug price control measures that could be enacted during the 2019 budget process or in other future legislation, including, for example, measures to permit Medicare Part D plans to negotiate the price of certain drugs under Medicare Part B, to allow some states to negotiate drug prices under Medicaid, and to eliminate cost sharing for generic drugs for low-income patients. Additionally, on May 11, 2018, the current U.S. presidential administration laid out the administration's "Blueprint" to reduce the cost of prescription medications while preserving innovation and cures. While the Department of Health and Human Services, or HHS, is soliciting feedback on some of these measures, other actions may be immediately implemented by HHS under existing authority. Although a number of these, and other potential, proposals will require authorization through additional legislation to become effective, Congress and the current U.S. presidential administration have each indicated that it will continue to seek new legislative and/or administrative measures to control drug costs. At the state level, legislatures are increasingly passing legislation and implementing regulations designed to control pharmaceutical and biological product pricing, including price or patient reimbursement constraints, discounts, restrictions on certain product access and marketing cost disclosure and transparency measures, and, in some cases, designed to encourage importation from other countries and bulk purchasing.

Additionally, on May 30, 2018, the Trickett Wendler, Frank Mongiello, Jordan McLinn, and Matthew Bellina Right to Try Act of 2017 was signed into law. The law, among other things, provides a federal framework for certain patients to access certain investigational new drug products that have completed a Phase I clinical trial and that are undergoing investigation for FDA approval. Under certain circumstances, eligible patients can seek treatment without enrolling in clinical trials and without obtaining FDA authorization under an FDA expanded access program; however, manufacturers are not obligated to provide investigational new drug products under the current federal right to try law.

Employees

As of June 7, 2019, we had 44 full-time employees and five part-time contract employees. Of these employees, 21 have an M.D. or Ph.D. None of our employees are represented by a labor union or covered by collective bargaining agreements, and we believe our relationship with our employees is good.

Facilities

We currently occupy approximately 23,000 square feet of office and laboratory space in Bedford, Massachusetts, under a lease that expires in 2021. The Bedford facility can accommodate at least 75 full-time employees. We have also signed a lease for an additional 2,485 square feet of office space in Cambridge, Massachusetts that expires in 2022, and we occupied this space in May 2019. The Cambridge office can accommodate at least 14 full-time employees. We believe that our facilities suffice to meet our current and near-term needs and that suitable additional space will be available as and when needed.

Legal proceedings

From time to time, we may be involved in legal proceedings arising in the ordinary course of our business. We are not presently a party to any legal proceedings that, in the opinion of management, would have a material adverse effect on our business. Regardless of outcome, litigation can have an adverse impact on us due to defense and settlement costs, diversion of management resources, negative publicity and reputation harm, and other factors.

Management

Executive officers, key employees and directors

The following table provides information regarding our executive officers, key employees and directors as of June 7, 2019:

Name	Age	Position
Executive officers:		
Edward M. Kaye, M.D.	70	Chief Executive Officer and Director
Huw M. Nash, Ph.D.	52	Chief Operating Officer and Chief Business Officer
Stephen J. Tulipano, CPA	60	Chief Financial Officer
Barry S. Ticho, M.D., Ph.D., FACC	59	Chief Medical Officer
Gene Liau, Ph.D.	64	Executive Vice President, Head of Research and Preclinical Development
Key employees:		
Isabel Aznarez, Ph.D.	47	Vice President of Biology
Charles R. Allerson, Ph.D.	51	Vice President of Chemistry
Dawn Kalmar	42	Vice President, Head of Corporate Affairs
Meena, Ph.D.	46	Vice President of Bioanalytical, DMPK and Biomarker Development
Shamim Ruff	59	Senior Vice President of Regulatory Affairs and Quality
Nancy M. Wyant	47	Vice President and Head of Clinical Operations
Non-employee directors:		
Jennifer C. Burstein(1)	47	Director
Adrian R. Krainer, Ph.D.(3)	60	Director
Arthur A. Levin, Ph.D.(1)(2)(3)	65	Director
Seth L. Harrison, M.D.(2)(3)	58	Director
Samuel W. Hall, Ph.D.	37	Director
Arthur O. Tzianabos, Ph.D.(1)(2)	56	Director

(1) Member of the Audit Committee.

(2) Member of the Compensation Committee.

(3) Member of the Nominating and Governance Committee.

Executive officers

Edward M. Kaye, M.D., has served as our Chief Executive Officer and a member of our board of directors since October 2017. Dr. Kaye joined us from Sarepta Therapeutics, Inc., a medical research and drug development company, where he served as President and Chief Executive Officer from September 2016 to July 2017, Interim Chief Executive Officer from April 2015 to September 2016 and Chief Medical Officer from June 2011 to March 2017. From 2001 to 2007, Dr. Kaye served in various positions at Genzyme Corporation, a biotechnology company, including most recently as Group Vice President of Clinical Development. Previously, Dr. Kaye served

as Chief of Biochemical Genetics at Children's Hospital of Philadelphia, Chief of Neurology at St. Christopher's Hospital for Children, and as a member of the research staff at Massachusetts General Hospital and Tufts University Medical Center. Dr. Kaye currently serves as a Neurological Consultant at the Children's Hospital of Boston. Dr. Kaye is also a member of the boards of directors of Cytokinetics, Inc., a public biopharmaceutical company, and the Massachusetts Biotechnology Council, a private non-profit life sciences industry organization. Dr. Kaye holds a B.S. in Biology/Chemistry from Loyola University and a M.D. from the Loyola University Stritch School of Medicine. We believe that Dr. Kaye is qualified to serve on our board of directors because of his extensive leadership and clinical experience in the medical and biotechnology fields.

Huw M. Nash, Ph.D., has served as our Chief Operating Officer and Chief Business Officer since October 2017, and served as our Chief Executive Officer from October 2014 to October 2017. Dr. Nash also serves as an Entrepreneur-in-Residence at Apple Tree Partners, a venture capital firm. While at Apple Tree Partners, Dr. Nash was part of the founding group of entrepreneurs who worked on the original platform technology for Aileron Therapeutics, Inc., a biopharmaceutical company. Dr. Nash also served as Vice President of Corporate Development at Aileron Therapeutics, Inc. from 2005 to 2013. Prior to joining Aileron Therapeutics, Inc., Dr. Nash was a founding scientist of NeoGenesis Pharmaceuticals, Inc., a drug discovery company, where he served as Vice President of External Collaborations from 1997 to 2005 until its acquisition by Schering-Plough Corp. Dr. Nash holds a B.A. in Biochemical Sciences from Harvard College and a Ph.D. in Organic Chemistry from Harvard University.

Stephen J. Tulipano, CPA, has served as our Chief Financial Officer since March 2019. From June 2014 to July 2018, Mr. Tulipano served as Chief Financial Officer and Treasurer of Aldeyra Therapeutics, Inc., a biotechnology company. From January 2011 to June 2014, Mr. Tulipano served in an accounting and management advisory role at Three Tulips, Inc. Previously, Mr. Tulipano was Chief Financial Officer and Secretary of Javelin Pharmaceuticals, Inc. Mr. Tulipano holds a B.S. from Salem State College and an M.B.A. from Suffolk University. He is a Certified Public Accountant.

Barry S. Ticho, M.D., Ph.D., FACC, has served as our Chief Medical Officer since October 2017. From February 2016 to September 2017, Dr. Ticho served as Head of Cardiovascular and Metabolic Diseases at Moderna, Inc., a biotechnology company. From October 2013 to February 2016, Dr. Ticho served as Head of External Research and Development Innovation for the Cardiovascular and Metabolic Disease Research Unit at Pfizer, Inc., a pharmaceutical company. Previously, Dr. Ticho served as the Vice President of Clinical Development at Biogen Inc., a biopharmaceutical company. Dr. Ticho holds a B.A. in Biology from Haverford College and an M.D. and a Ph.D. in Biochemistry and Molecular Biology from the University of Chicago.

Gene Liau, Ph.D., has served as our Executive Vice President, Head of Research and Preclinical Development since January 2018. From September 2015 to August 2017, Dr. Liau served as Senior Vice President and Head of Gene Therapy Research and Development at Precision Biosciences, Inc., a biotechnology company. From July 2011 to June 2015, Dr. Liau served in various roles at Pfizer, Inc., a pharmaceutical company, most recently as Executive Director and Head of External Research and Development Rare Diseases and Hematology. Dr. Liau holds a B.S. in Biology from the University of North Carolina at Chapel Hill and a Ph.D. in Biochemistry from Vanderbilt University.

Key employees

Isabel Aznarez, Ph.D., co-founded Stoke Therapeutics, Inc. and has served as our Vice President of Biology since October 2014. Prior to co-founding Stoke Therapeutics, Inc., Dr. Aznarez served as a Postdoctoral Fellow, then Research Investigator, at Cold Spring Harbor Laboratory, a biological research institution, from January 2008 to November 2015. Dr. Aznarez also serves on the board of directors of the Oligonucleotide Therapeutics Society. Dr. Aznarez holds a B.S. in Biology and Human Genetics from the University of Uruguay and a Ph.D. in Molecular Genetics from the University of Toronto.

Charles R. Allerson, Ph.D., has served as our Vice President of Chemistry since December 2017. Prior to joining us, Dr. Allerson served as Associate Director and then Director of Chemistry at Regulus Therapeutics Inc., a microRNA-focused biopharmaceutical company, from August 2010 to December 2017, and also as a Senior Scientist and Principal Scientist at Ionis Pharmaceuticals Inc., a RNA-focused biopharmaceutical company, from August 2002 to August 2010. Dr. Allerson holds a B.S. in Chemistry from Lafayette College and a Ph.D. in Chemistry from Harvard University.

Dawn Kalmar has served as our Vice President, Head of Corporate Affairs since April 2019. From May 2014 to January 2018, Ms. Kalmar served as Vice President, Corporate Communications at Vertex Pharmaceuticals, Inc., a biopharmaceutical company. From September 2010 to May 2014, Ms. Kalmar served in various roles at Vertex Pharmaceuticals, Inc., including leading Internal Communications and Employee Engagement from October 2012 to May 2014 and, prior to that, leading Product Communications from September 2010 to October 2012. From April 2003 to August 2010, Ms. Kalmar held roles of increasing responsibility for Internal and External Communications and Patient Advocacy for Genentech, Inc., a biotechnology company. Ms. Kalmar holds a B.S. in Journalism from California Polytechnic State University, San Luis Obispo, California.

Meena, Ph.D., has served as our Vice President of Bioanalytical, DMPK and Biomarker Development since April 2018. Prior to joining us, Ms. Meena served at Wave Life Sciences Ltd., a biopharmaceutical company, from March 2010 to March 2018, where most recently she was Senior Director of Bioanalytical, Pharmacology, and Biomarker Development. From July 2006 to January 2010, Ms. Meena served at Alnylam Pharmaceuticals, Inc., a biopharmaceutical company, as a scientist supporting drug discovery. Ms. Meena holds a B.S. from S.R. Govt. College for Women, B.Ed. from the D.A.V. College of Education for Women, Amritsar, an M.S. in Chemistry from Khalsa College, Amritsar, and a Ph.D. in Chemistry from the National Chemical Laboratory, Pune, India.

Shamim Ruff has served as our Senior Vice President of Regulatory Affairs and Quality since December 2018. Prior to joining us, Ms. Ruff led the Regulatory Affairs and Quality teams at Sarepta Therapeutics, Inc., a medical research and drug development company, where she was Chief Regulatory Affairs Officer and Senior Vice President of Quality from December 2015 to May 2018 and Vice President of Regulatory Affairs and Quality from January 2013 to November 2015. Prior to joining Sarepta Therapeutics, Inc., Ms. Ruff served as Vice President, Head of Global Regulatory Affairs Oncology at Sanofi Genzyme, a biotechnology company, and held senior positions at Amgen Inc., Abbott Laboratories Inc., and AstraZeneca PLC, where she oversaw the development and filings of multiple successful regulatory approvals across the world. Ms. Ruff holds a B.S. in Chemistry and Biology from the University of Leicester, UK and an MSc. in Analytical Chemistry from the University of Loughborough, UK.

Nancy M. Wyant has served as our Vice President and Head of Clinical Operations since December 2018. Prior to this appointment, Ms. Wyant served as an independent clinical program management and operations consultant for us from February 2018 to December 2018. From January 2017 to January 2018, Ms. Wyant served as Vice President of Global Clinical Operations at BeiGene USA, Inc., a biopharmaceutical company. Prior to joining BeiGene USA, Inc., Ms. Wyant served as Vice President of Clinical Operations at Idera Pharmaceuticals, Inc., a biotechnology company, from May 2014 to June 2016, and as Head of Clinical Operations at Sarepta Therapeutics, Inc., a medical research and drug development company, from January 2013 to May 2014. Ms. Wyant holds a B.A. in Psychology from Hartwick College.

Non-employee directors

Jennifer C. Burstein, CPA, has served as a member of our board of directors since June 2019. From January 2018 to February 2019, Ms. Burstein served as Senior Vice President of Finance and principal financial officer at Loxo Oncology, Inc., a biotechnology company, until its acquisition by Eli Lilly and Company. Ms. Burstein previously served as Vice President of Finance and principal financial officer of Loxo Oncology since May 2015. Prior to Loxo Oncology, Ms. Burstein served as Vice President of Finance at Acorda Therapeutics, Inc., a public biotechnology company, from July 2010 until April 2015, where she held several positions of increasing

responsibility in Finance from 2006 until being appointed Vice President of Finance. Prior to joining Acorda, from 2002 to 2006, she was with Eyetech Pharmaceuticals, Inc., a public biotechnology company, which is currently a subsidiary of Valeant Pharmaceuticals International, Inc., where she held several positions of increasing responsibility in Finance until being promoted to Senior Director, Accounting. Before Eyetech, Ms. Burstein worked in the Finance departments at several companies and in public accounting. Ms. Burstein received her B.S. in Business Administration and M.B.A. in Accounting from the State University of New York at Buffalo and has a CPA license in New York. We believe that Ms. Burstein is qualified to serve on our board of directors because of her expertise in financial and accounting matters and her extensive experience in the biotechnology industry.

Adrian R. Krainer, Ph.D., co-founded Stoke Therapeutics, Inc. and has served as a member of our board of directors since June 2014. Professor Krainer is the St. Giles Professor at Cold Spring Harbor Laboratory, a biological research institution, where he has served since 1986 and where his work led directly to the invention and development of SPINRAZA. Professor Krainer holds a B.A. in Biochemistry from Columbia University and a Ph.D. in Biochemistry from Harvard University. We believe that Professor Krainer is qualified to serve on our board of directors because of his extensive experience in biopharmaceutical research and development and experience in RNA splicing and antisense therapies.

Arthur A. Levin, Ph.D., has served as a member of our board of directors since September 2015. Since January 2014, Dr. Levin has served as Executive Vice President of Research and Development at Avidity Biosciences LLC, a biotechnology company. From April 2012 to January 2014, Dr. Levin served as Executive Vice President at miRagen Therapeutics, Inc., an RNA-focused therapeutics company. Prior to joining miRagen Therapeutics, Inc., Dr. Levin served in various senior management positions at Santaris Pharma A/S Corp., a biopharmaceutical company, and Ionis Pharmaceuticals, Inc., a public, RNA-focused biopharmaceutical company. Dr. Levin holds a B.S. in Biology from Muhlenberg College and a Ph.D. in Toxicology from the University of Rochester. We believe that Dr. Levin is qualified to serve on our board of directors because of his industry experience, including his expertise in nucleic-acid-based therapeutics.

Seth L. Harrison, M.D., has served as the chairman of our board of directors since July 2015. Dr. Harrison serves as Managing Partner at Apple Tree Partners, a venture capital firm, which he founded in 1999. Prior to founding Apple Tree Partners, Dr. Harrison served as a General Partner at Oak Investment Partners, a venture capital and private equity firm, and as a Venture Partner at Sevin Rosen Funds, a technology-focused venture capital firm. Dr. Harrison currently serves as the chairman of the boards of directors of Braeburn Pharmaceuticals, Inc., a pharmaceutical company, Elstar Therapeutics, Inc., an immunotherapy company, and Limelight Bio, Inc., a gene therapy company, as well as several private companies. Dr. Harrison holds an A.B. from Princeton University and an M.D. and M.B.A. from Columbia University. We believe that Dr. Harrison is qualified to serve on our board of directors because of his experience in the life sciences industry, his experience as a venture capitalist, as well as his service on the boards of directors of numerous biopharmaceutical companies.

Samuel W. Hall, Ph.D., has served as a member of our board of directors since July 2015. Dr. Hall serves as a Partner at Apple Tree Partners, a venture capital firm. From October 2008 until joining Apple Tree Partners in April 2013, Dr. Hall served as a researcher at the University of Cambridge. Previously, Dr. Hall served on the investment team at Symphony Capital, a private equity firm dedicated to investments in biopharmaceutical development, and a member of the healthcare investment banking team at Citigroup Inc., a multinational investment bank. Dr. Hall currently serves on the boards of directors of Elstar Therapeutics, Inc., an immunotherapy company, Limelight Bio, Inc., a gene therapy company, Chinook Therapeutics, Inc., a biotechnology company, and the Burke Neurological Institute, a non-profit research institute. Dr. Hall previously served on the board of directors of Syntimmune, Inc., a biotechnology company, prior to its acquisition by Alexion Pharmaceuticals, Inc. in November 2018. Dr. Hall holds an A.B. in Molecular Biology from Princeton University and an M.Phil. and Ph.D. from the University of Cambridge. We believe that Dr. Hall is

qualified to serve on our board of directors because of his experience working with and serving on the boards of directors of various life sciences companies and his experience as a venture capitalist.

Arthur O. Tzianabos, Ph.D., has served as a member of our board of directors since September 2018. Since April 2016, Dr. Tzianabos has served as President and Chief Executive Officer at Homology Medicines, Inc., a genetic medicines company. Prior to joining Homology Medicines, Inc., Dr. Tzianabos served as President and Chief Scientific Officer at OvaScience, Inc., a biotechnology company, from September 2013 to March 2016. Previously, Dr. Tzianabos served in various senior management positions at Shire Plc, a pharmaceutical company, and as a professor at Harvard Medical School. Dr. Tzianabos serves on the board of directors of Akouos, Inc., a private biotechnology company. Dr. Tzianabos holds a B.S. in Biology from Boston College and a Ph.D. in Microbiology from the University of New Hampshire. We believe that Dr. Tzianabos is qualified to serve on our board of directors because of his executive and clinical experience, as well as his extensive involvement in the biotechnology industry.

Election of officers

Our executive officers are appointed by, and serve at the discretion of, our board of directors. There are no family relationships among any of our directors or executive officers.

Board composition

Our board of directors currently consists of seven members. Six of our directors are independent within the meaning of the independent director guidelines of Nasdaq. Pursuant to our current voting agreement and certificate of incorporation, Adrian R. Krainer, Seth L. Harrison, Samuel W. Hall, Arthur A. Levin, Arthur O. Tzianabos, Jennifer C. Burstein and Edward M. Kaye have been designated to serve as members of our board of directors. Adrian R. Krainer and Edward M. Kaye were elected by the holders of our common stock and are representatives of the common shareholders. Seth L. Harrison and Samuel W. Hall were elected by the holders of our Series A convertible preferred stock and are representatives of the preferred shareholders. Arthur A. Levin was elected by the holders of our common stock and the Series A convertible preferred stock, voting together as a single class on an as-converted basis. Arthur O. Tzianabos and Jennifer C. Burstein were elected by the holders of our common stock and convertible preferred stock, voting together as a single class on an as-converted basis.

Arthur A. Levin and Arthur O. Tzianabos, were mutually agreed upon to serve on the board of directors by the representatives of the common shares and the representatives of the preferred shares. Jennifer C. Burstein was nominated by the full board of directors and mutually agreed upon by the holders of a majority of the Series B convertible preferred stock. Adrian R. Krainer, Edward M. Kaye, Jennifer C. Burstein, Arthur A. Levin and Arthur O. Tzianabos do not own preferred shares and are not affiliated with, and are independent of, the preferred shareholders.

The voting agreement and the provisions of our current certificate of incorporation that govern the election and designation of our directors will terminate in connection with this offering, after which no contractual obligations will concern the election of our directors. Each of our current directors will continue to serve until the election and qualification of his or her successor, or until his or her earlier death, resignation or removal.

Classified board of directors

Upon the completion of this offering, our board of directors will be divided into three staggered classes of directors. At each annual meeting of stockholders, a class of directors will be subject to re-election for a three-year term. As a result, only one class of directors will be elected at each annual meeting of our stockholders, with the other classes continuing for the remainder of their respective three-year terms. Our directors will be divided among the three classes as follows:

- the Class I directors will be Samuel W. Hall and Adrian R. Krainer and their terms will expire at the first annual meeting of stockholders held following the completion of the offering;

- the Class II directors will be Jennifer C. Burstein and Arthur A. Levin and their terms will expire at the second annual meeting of stockholders held following the completion of the offering; and
- the Class III directors will be Edward M. Kaye, Seth L. Harrison and Arthur Tzianabos and their terms will expire at the third annual meeting of stockholders held following the completion of the offering.

Each director's term continues until the election and qualification of his or her successor, or his or her earlier death, resignation or removal. Our restated certificate of incorporation and restated bylaws that will be in effect upon the completion of this offering authorize only our board of directors to fill vacancies on our board of directors. Any increase or decrease in the number of directors will be distributed among the three classes so that, as nearly as possible, each class will consist of one-third of the directors. This classification of our board of directors may have the effect of delaying or preventing changes in control of our company. See the section entitled "Description of capital stock—Anti-takeover provisions—Restated certificate of incorporation and restated bylaw provisions."

Director independence

After the completion of this offering, an entity affiliated with Apple Tree Partners will beneficially own a majority of the voting power of all outstanding shares of our common stock. As a result, after the filing of our restated certificate of incorporation and the automatic termination of the amended and restated voting agreement as of the closing of this offering, we will be a "controlled company" within the meaning of the corporate governance standards of Nasdaq. We will remain a "controlled company" so long as more than 50% of the voting power for the election of directors is held by Apple Tree Partners. However, we do not intend to avail ourselves of the controlled company exemptions under the Nasdaq listing rules.

Under the rules of Nasdaq, independent directors must comprise a majority of a listed company's board of directors within a specified period following the completion of this offering. In addition, the rules of Nasdaq require that, subject to specified exceptions, each member of a listed company's audit, compensation and nominating and governance committees be independent. Under the rules of Nasdaq, a director will only qualify as an "independent director" if, in the opinion of that company's board of directors, that person does not have a relationship that would interfere with the exercise of independent judgment in carrying out the responsibilities of a director.

Audit committee members must also satisfy the independence criteria set forth in Rule 10A-3 under the Securities Exchange Act of 1934, as amended, or the Exchange Act. In order to be considered independent for purposes of Rule 10A-3, a member of an audit committee of a listed company may not, other than in his or her capacity as a member of the audit committee, the board of directors or any other board committee: (i) accept, directly or indirectly, any consulting, advisory or other compensatory fee from the listed company or any of its subsidiaries; or (ii) be an affiliated person of the listed company or any of its subsidiaries. We intend to satisfy the audit committee independence requirements of Rule 10A-3 as of the completion of this offering. Additionally, compensation committee members must not have a relationship with us that is material to the director's ability to be independent from management in connection with the duties of a compensation committee member.

Our board of directors has undertaken a review of the independence of each director and considered whether each director has a material relationship with us that could compromise his or her ability to exercise independent judgment in carrying out his or her responsibilities. As a result of this review, our board of directors determined that all of our directors, except for Edward M. Kaye, M.D. are "independent directors" as defined under the applicable rules and regulations of the SEC and the listing requirements and rules of Nasdaq. In making these determinations, our board of directors reviewed and discussed information provided by the directors and us with regard to each director's business and personal activities and relationships as the may relate to us and our management, including the beneficial ownership of our capital stock by each non-employee director and then transactions involving them described in the section entitled "Certain relationships and related party transactions."

Committees of the board of directors

Our board of directors has an audit committee, a compensation committee and a nominating and governance committee, each of which will have the composition and responsibilities described below as of the completion of this offering. Each of the below committees has a written charter approved by our board of directors. Upon completion of this offering, copies of each charter will be posted on the investor relations section of our website. Members serve on these committees will serve until their resignation or until otherwise determined by our board of directors.

Audit committee

Our audit committee is comprised of Arthur Tzianabos, Arthur A. Levin and Jennifer C. Burstein , with Jennifer C. Burstein serving as the chairperson of our audit committee. The composition of our audit committee meets the requirements for independence under the current Nasdaq listing standards and SEC rules and regulations. Each member of our audit committee is financially literate. In addition, our board of directors has determined that Jennifer C. Burstein is an "audit committee financial expert" as defined in Item 407(d)(5)(ii) of Regulation S-K promulgated under the Securities Act of 1933, as amended. This designation does not impose on him or her any duties, obligations or liabilities that are greater than are generally imposed on members of our audit committee and our board of directors. Our audit committee is directly responsible for, among other things:

- selecting and hiring our independent registered public accounting firm;
- the qualifications, independence and performance of our independent auditors;
- the preparation of the audit committee report to be included in our annual proxy statement;
- our compliance with legal and regulatory requirements;
- our accounting and financial reporting processes, including our consolidated financial statement audits and the integrity of our consolidated financial statements; and
- reviewing and approving related-person transactions.

Compensation committee

Our compensation committee is comprised of Seth L. Harrison, Arthur Tzianabos and Arthur A. Levin, with Arthur Tzianabos serving as the chairman of our compensation committee. Each member of our compensation committee is a non-employee director, as defined by Rule 16b-3 promulgated under the Exchange Act and meets the requirements for independence under the current Nasdaq listing standards and SEC rules and regulations. Our compensation committee is responsible for, among other things:

- evaluating, recommending, approving and reviewing executive officer compensation arrangements, plans, policies and programs;
- evaluating and recommending non-employee director compensation arrangements for determination by our board of directors;
- administering our cash-based and equity-based compensation plans; and
- overseeing our compliance with regulatory requirements associated with the compensation of directors, officers and employees.

Nominating and governance committee

Our nominating and governance committee is comprised of Seth L. Harrison, Arthur A. Levin and Adrian Krainer, with Seth L. Harrison serving as the chairman of our nominating and governance committee. Each

member of our nominating and governance committee meets the requirements for independence under the current Nasdaq listing standards. Our nominating and governance committee is responsible for, among other things:

- identifying, considering and recommending candidates for membership on our board of directors;
- overseeing the process of evaluating the performance of our board of directors; and
- advising our board of directors on other corporate governance matters.

Compensation committee interlocks and insider participation

None of the members of our compensation committee has at any time been one of our officers or employees, and none of our executive officers has served as a member of the board of directors, or as a member of the compensation or similar committee, of any entity that has one or more executive officers who served on our board of directors or compensation committee during the year ended December 31, 2018. Prior to establishing the compensation committee, our full board of directors made decisions relating to the compensation of our officers.

Code of business conduct and ethics

Prior to the completion of this offering, our board of directors will adopt a code of business conduct and ethics that applies to all of our employees, officers and directors, including our Chief Executive Officer, Chief Financial Officer and other executive and senior officers. The full text of our code of business conduct and ethics will be posted on the investor relations section of our website. The reference to our website address in this prospectus does not include or incorporate by reference the information on or accessible through our website into this prospectus. We intend to disclose future amendments to certain provisions of our code of business conduct and ethics, or waivers of these provisions, on our website or in public filings to the extent required by the applicable rules.

Non-employee director compensation

The following table presents the total compensation earned by each of our non-employee directors in the year ended December 31, 2018. Our Chief Executive Officer, Dr. Kaye, receives no compensation for his service as a director. Other than as described below, none of our non-employee directors received any fees or reimbursement of any expenses (other than customary expenses in connection with the attendance of meetings of our board of directors) or any equity or non-equity awards in the year ended December 31, 2018.

Name	Fees earned or paid in cash ($)	Option awards ($)(1)(2)	All other compensation ($)	Total ($)
Adrian R. Krainer, Ph.D.	—	88,734	100,000[3]	188,734
Arthur A. Levin, Ph.D.	30,000	36,536	—	66,536
Seth L. Harrison, M.D.	—	—	—	—
Samuel W. Hall, Ph.D.	—	—	—	—
Arthur O. Tzianabos, Ph.D.	7,500	79,206	—	86,706

(1) The amounts reported in this column represent the aggregate grant date fair value of the awards granted under our 2014 Plan to our directors during the year ended December 31, 2018 as computed in accordance with FASB ASC Topic 718. The assumptions used in calculating the grant date fair value of the awards reported in the Option Awards column are set forth in Note 10 to our consolidated financial statements included elsewhere in this prospectus. Note that the amounts reported in this column reflect the aggregate accounting cost for these awards, and do not necessarily correspond to the actual economic value that may be received by the director from the awards.

(2) The following table sets forth the aggregate number of shares of our common stock subject to outstanding options held by our non-employee directors as of December 31, 2018:

Director name	Number of shares underlying options held as of December 31, 2018(1)
Adrian R. Krainer, Ph.D.(2)	217,711
Arthur A. Levin, Ph.D.(3)	43,754
Seth L. Harrison, M.D.	—
Samuel W. Hall, Ph.D.	—
Arthur O. Tzianabos, Ph.D.(4)	61,739

(1) All of the outstanding equity awards were granted under our 2014 Plan.

(2) This amount reflects (i) options to purchase 149,112 shares, all of which are fully vested and (ii) options to purchase 68,599 shares, 1/48th of which vest monthly following the October 22, 2018 vesting commencement date.

(3) This amount reflects (i) options to purchase 2,775 shares, 1/48th of which vest monthly following the October 6, 2015 vesting commencement date, (ii) options to purchase 1,841 shares, 1/48th of which vest monthly following the August 1, 2016 vesting commencement date, (iii) options to purchase 16,645 shares, 1/48th of which vest monthly following the January 31, 2018 vesting commencement date and (iv) options to purchase 22,493 shares, 1/48th of which vest monthly following the October 22, 2018 vesting commencement date.

(4) This amount reflects options to purchase 61,739 shares, 1/48th of which vest monthly following the September 4, 2018 vesting commencement date.

(3) Represents fees paid to Professor Krainer pursuant to Professor Krainer's consulting agreement. See "Certain relationships and related party transactions—Consulting agreement."

Prior to this offering, we did not have a formal policy to provide any cash or equity compensation to our non-employee directors for their service on our board of directors or committees of our board of directors.

In June 2019, our board of directors approved compensation for our non-employee directors, to be effective upon completion of this offering.

Non-Employee Director Cash Compensation

Beginning after this offering, our non-employee directors will receive annual cash compensation of $35,000 for service on the board of directors, and additional cash compensation for the chairperson and committee members as set forth below. All cash payments will be made quarterly in arrears and will be pro-rated for any partial quarters of service.

- Board Chairperson: $30,000
- Audit Committee Chair: $15,000
- Audit Committee Member (Non-Chair): $7,500
- Compensation Committee Chair: $10,000
- Compensation Committee Member (Non-Chair): $5,000
- Nominating and Corporate Governance Committee Chair: $8,000
- Nominating and Corporate Governance Committee Member (Non-Chair): $4,000

Non-Employee Director Equity Grants

Initial public offering option grant

Following this offering, each non-employee director who is serving on the board of directors at the completion of the offering and who continues to serve on the board of directors at the date of grant will be granted 29,555 shares of our common stock, referred to as the Initial IPO Grant. Each Initial IPO Grant will vest in 12 substantially equal installments on each quarterly anniversary of the grant date, subject to the director's continued service on each applicable vesting date.

Initial appointment option grant

In addition, each non-employee director who is elected or appointed to our board of directors after completion of this offering will, unless the board of directors determines that such grant will not be made automatically, be automatically granted an option to purchase 29,555 shares of our common stock upon the director's initial appointment to our board of directors, referred to as the Initial Grant. The Initial Grant will vest in 12 substantially equal quarterly installments on each quarterly anniversary of the date of grant, such that the Initial Grant will become fully vested and exercisable on the three-year anniversary of the date of grant, subject to the director's continued service on each applicable vesting date.

Annual option grant

Each non-employee director who is serving on our board of directors immediately prior to, and will continue to service on the Board following, our annual meeting of stockholders, will, unless the board of directors determines that such grant will not be made automatically, be automatically granted an option to purchase 14,777 shares of our common stock on the date of such annual meeting of stockholders, referred to as the Annual Grant. Each Annual Grant will vest on the one-year anniversary of the date of grant, such that the Annual Grant will become fully vested and exercisable on the one-year anniversary of the date of grant, or if earlier, the next annual meeting of stockholders, subject to the director's continued service on the applicable vesting date.

The Initial IPO Grants, the Initial Grants and the Annual Grants will be subject to the terms and conditions of the 2019 Equity Incentive Plan and will fully vest and become exercisable upon the consummation of a corporate transaction (as defined in our 2019 Equity Incentive Plan and pursuant to the terms of our 2019 Equity Incentive Plan).

Executive compensation

The following tables and accompanying narrative disclosure set forth information about the compensation earned by our named executive officers during the year ended December 31, 2018. Our named executive officers, who are our principal executive officer and the two most highly-compensated executive officers (other than our principal executive officer) serving as executive officers as of December 31, 2018, were:

- Edward M. Kaye, M.D., Chief Executive Officer and Director;
- Huw M. Nash, Ph.D., Chief Operating Officer and Chief Business Officer; and
- Barry S. Ticho, M.D., Ph.D., FACC, Chief Medical Officer.

Summary compensation table

The following table presents summary information regarding the total compensation for services rendered in all capacities that was awarded to and earned by our named executive officers during the year ended December 31, 2018:

Name and principal position	Salary($)	Non-equity incentive plan compensation ($) (1)	Option awards ($)(2)	Total ($)
Edward M. Kaye, M.D. *Chief Executive Officer*	463,500	185,400	837,748	1,486,648
Huw M. Nash, Ph.D. *Chief Operating Officer and Chief Business Officer*	318,270	111,395	440,190	869,855
Barry S. Ticho, M.D., Ph.D., FACC *Chief Medical Officer*	360,500	140,650	239,940	741,090

(1) For additional information regarding non-equity incentive plan compensation, see the section entitled “Non-equity incentive plan.”
(2) Represents the grant date fair value of options awarded during the year ended December 31, 2018 as computed in accordance with FASB ASC Topic 718. The assumptions used in calculating the grant date fair value of the stock options reported in the Options Award column are set forth in Note 10 to our consolidated financial statements included elsewhere in this prospectus. Note that the amounts reported in this column reflect the aggregate accounting cost for these awards, and do not necessarily correspond to the actual economic value that may be received by each named executive officer from the options.

Non-equity incentive plan

Annual bonuses for our executive officers are based on the achievement of corporate and individual performance objectives. In February 2019, based on the achievement of these goals with respect to 2018 performance, our board of directors determined to award bonuses equal to 100% of target.

Outstanding equity awards at 2018 fiscal year-end table

	Option awards					
Name	Grant date(1)	Vesting commencement date	Number of securities underlying unexercised options exercisable	Number of securities underlying unexercised options unexercisable	Option exercise price ($)	Option expiration date
Edward M. Kaye	4/2/2018(2)	10/17/2017	203,524	494,277	0.60	4/2/2028
	12/12/2018(3)	10/22/2018	19,515	448,867	2.19	12/12/2028
Huw M. Nash	2/11/2016(4)	12/1/2015	152,324	—	0.40	2/11/2026
	2/2/2017(5)	8/1/2016	29,413	21,010	0.40	2/2/2027
	4/2/2018(5)	1/31/2018	59,344	199,613	0.60	4/2/2028
	12/12/2018(5)	10/22/2018	11,399	262,198	2.19	12/12/2028
Barry S. Ticho	4/2/2018(2)	10/2/2017	63,318	153,775	0.60	4/2/2028
	12/12/2018(3)	10/22/2018	5,360	123,284	2.19	12/12/2028

(1) All of the outstanding equity awards were granted under the 2014 Plan.

(2) 1/4th of the option vested on the one-year anniversary of the vesting commencement date and an additional 1/48th vests monthly thereafter, subject to the executive's continued service to us. The options are also subject to double trigger acceleration as described in greater detail in the section entitled "Potential payments upon termination or change in control."

(3) 1/48th of the option vests on each one-month anniversary of the vesting commencement date, subject to the executive's continued service to us. The options are also subject to double trigger acceleration as described in greater detail in the section entitled "Potential payments upon termination or change in control."

(4) The option is 100% vested.

(5) 1/48th of the option vests on each one-month anniversary of the vesting commencement date, subject to the executive's continued service to us. 100% of the shares subject to the option will vest upon a "change in control" as defined in Dr. Nash's option agreements and **as** described in greater detail in the section entitled "Potential payments upon termination or change in control."

Employment agreements

We have entered into employment agreements with each of our named executive officers that provide for "at-will" employment and include each named executive officer's base salary, target discretionary annual incentive bonus opportunity, and initial option grant, or Initial Option Grant. These agreements also provide for severance benefits upon certain involuntary terminations of employment, as described below.

Pursuant to the employment agreements, upon a termination of each named executive officer's employment without "cause" or for "good reason" (each as defined in the applicable executive's employment agreement and as described below), subject to the executive's execution and non-revocation of a release of claims in favor of the company, the executive will be entitled to continued salary payments and COBRA reimbursement for 12 months following termination of employment, in the case of Drs. Nash and Kaye, and six months following termination of employment, in the case of Dr. Ticho.

Each named executive officer is also entitled to his earned but unpaid bonus upon a termination for any reason other than for cause.

Additionally, upon a termination without cause or for good reason within the 90-day period prior to the execution of a definitive agreement providing for the consummation of a "change in control" (as defined in the employment agreement) or the one-year period following a change in control, Drs. Kaye and Ticho will be entitled to full acceleration of the Initial Option Grant.

Pursuant to the employment agreements, each named executive officer is also subject to a post-termination non-competition covenant that extends for 12 months following termination of employment, in the case of Drs. Kaye and Nash, and six months following termination of employment, in the case of Dr. Ticho. In the event the named executive officer breaches his non-competition covenant, such executive will forfeit any unpaid severance benefits.

We have also entered into employee invention assignment and confidentiality agreements with each of our named executive officers, which agreements include a 12-month post-termination non-solicitation covenant.

For purposes of the employment agreements "cause" generally means:

- the executive willfully engages in conduct that is in bad faith and materially injurious to us, including but not limited to, misappropriation of trade secrets, fraud or embezzlement;
- the executive commits a material breach of any written agreement between the executive and us that causes harm to the company, which breach is not timely cured;
- the executive willfully refuses to implement or follow a directive by the board of directors, directly related to the executive's duties, which breach is not timely cured; or
- the executive engages in material misfeasance or malfeasance demonstrated by a continued pattern of material failure to perform the essential job duties associated with executive's position, which breach is not timely cured.

For purposes of the employment agreements "good reason" generally means:

- a material reduction in the executive's duties or responsibilities that is inconsistent with the executive's position;

- the requirement that the executive change his principal office to a facility that increases his commute by more than 40 miles from his commute to the location at which he was employed prior to such change, or
- a material reduction in the executive's base salary or a material reduction in the executive's employee benefits.

Potential payments upon termination or change in control

Employment Agreements

As described in greater detail above in the section entitled "—Employment agreements", each named executive officer is eligible to receive certain severance and/or acceleration benefits upon a qualifying termination of employment pursuant to such named executive officer's employment agreement.

Option Grant Agreements

Pursuant to Dr. Nash's option grant agreements with us, Dr. Nash is also entitled to receive acceleration of vesting of 100% of the shares subject to each of his outstanding and unvested options upon a change in control.

Pursuant to each of Dr. Kaye's and Dr. Ticho's option grant agreements with us, upon a termination of the executive's employment without cause or for good reason (each as defined above under the section entitled "—Employment agreements") within the period beginning 90 days prior to execution of a definitive agreement providing for the consummation of a change in control and ending 12 months following a change in control, the executive will be entitled to 100% acceleration of any outstanding and unvested options subject to such option grant agreements.

For purposes of the option grant agreements, change in control generally means (i) a transaction or series of transactions resulting in our liquidation, dissolution or winding up, (ii) a sale of all or substantially all of our assets, followed by a liquidation, dissolution or winding up, (iii) any sale or exchange of our capital stock by our stockholders where more than 50% of the outstanding voting power of the company is acquired by a person or entity or group of related persons or entities (other than pursuant to a recapitalization solely with our equity holders), or (iv) any merger or consolidation in which our pre-merger or pre-consolidation shareholders do not possess at least a majority of the total voting power of the surviving entity.

Equity compensation plans and other benefit plans

2014 equity incentive plan

In 2014, we adopted the 2014 Equity Incentive Plan, or the 2014 Plan, as most recently amended on October 15, 2018. The purposes of the 2014 Plan are to attract and retain the best available personnel for positions of substantial responsibility, to provide additional incentive to employees, directors and consultants and to promote the success of our business. The material terms of the 2014 Plan are summarized below:

Share reserve. As of March 31, 2019, we had 4,652,098 shares of our common stock reserved for issuance pursuant to grants under our 2014 Plan of which 395,316 shares remained available for grant. As of March 31, 2019, options to purchase 222,133 shares had been exercised and options to purchase 4,034,649 of shares remained outstanding, with a weighted-average exercise price of $1.64 per share. As of March 31, 2019, no shares of restricted stock, no restricted stock units and no stock appreciation rights were granted under the 2014 Plan. No new awards will be granted under the 2014 Plan after the offering.

Administration. Our 2014 Plan is administered by our board of directors or a committee appointed by our board of directors, referred to herein as the "administrator". Subject to the terms of the 2014 Plan, the administrator has the authority to, among other things, select the persons to whom awards will be granted,

construe and interpret our 2014 Plan as well as to prescribe, amend and rescind rules and regulations relating to the 2014 Plan and awards granted thereunder. The administrator may modify awards subject to the terms of the 2014 Plan.

Eligibility. Pursuant to the 2014 Plan, we may grant incentive stock options only to our employees (including officers and directors who are also employees). We may grant non-statutory stock options and stock purchase rights to our employees (including officers and directors who are also employees), non-employee directors and consultants.

Options. The 2014 Plan provides for the grant of both (i) incentive stock options, which are intended to qualify for tax treatment as set forth under Section 422 of the Code, as amended, or the Code, and (ii) non-statutory stock options to purchase shares of our common stock, each at a stated exercise price. The exercise price of each option must be at least equal to the fair market value of our common stock on the date of grant (unless otherwise determined by the administrator). However, the exercise price of any incentive stock option granted to an individual who owns more than ten percent of the total combined voting power of all classes of our capital stock must be at least equal to 110% of the fair market value of our common stock on the date of grant.

The administrator will determine the vesting schedule applicable to each option. The maximum permitted term of options granted under our 2014 Plan is ten years from the date of grant, except that the maximum permitted term of incentive stock options granted to an individual who owns more than ten percent of the total combined voting power of all classes of our capital stock is five years from the date of grant.

Restricted stock, restricted stock units, stock appreciation rights. In addition, the 2014 Plan allows for the grant of restricted stock awards, restricted stock units and stock appreciation rights, with terms as generally determined by the administrator (in accordance with the 2014 Plan) and to be set forth in an award agreement. We have not granted any shares of restricted stock, any restricted stock units or any stock appreciation rights under the 2014 Plan.

Limited transferability. Unless otherwise determined by the Administrator, awards under the 2014 Plan generally may not be sold, pledged, assigned, hypothecated, transferred or disposed of in any manner other than by will, the laws of descent and distribution or qualified domestic relations orders.

Change of control. In the event that we are subject to an “acquisition” or “other combination” (as defined in the 2014 Plan and generally meaning, collectively, a merger, a sale or transfer of more than 50% of the voting power of all of our outstanding securities, or a sale of all or substantially all of the assets of ours), the 2014 Plan provides that awards will be subject to the agreement evidencing such acquisition or other combination, which agreement need not treat all awards in a similar manner. Such agreement may, without the participant’s consent, provide for the continuation of outstanding awards, the assumption or substitution of awards, the acceleration of vesting of awards, the settlement of awards (whether or not vested) in cash, securities or other consideration, or the cancellation of such awards for no consideration.

Adjustments. In the event of a dividend or other distribution, recapitalization, stock split, reverse stock split, reorganization, merger, consolidation, split-up, spin-off, combination, repurchase or exchange of any of our securities, or other change in our corporate structure affecting the shares of common stock issued under the 2014 Plan, the number and class of shares that may be delivered under 2014 Plan and/or the number, class and price of shares covered by each outstanding award will (to the extent appropriate) be appropriately adjusted (subject to required action by the board), in order to prevent diminution or enlargement of benefits or potential benefits intended to be made available under the 2014 Plan or otherwise as required by applicable law.

Exchange, repricing and buyout of awards. The administrator may, with the consent of the respective participants, issue new awards in exchange for the surrender and cancelation of any or all outstanding awards.

The administrator may also reduce the exercise price of options or stock appreciation rights or buy an award previously granted with payment in cash, shares or other consideration, in each case, subject to the terms of the 2014 Plan.

Amendment/termination. The Board may amend or terminate the 2014 Plan at any time and may terminate any and all outstanding options or stock appreciation rights upon a dissolution or liquidation of us, provided that certain amendments will require shareholder approval. We expect to terminate the 2014 Plan and will cease issuing awards thereunder upon the effective date of our 2019 Equity Incentive Plan (described below), which is the date immediately prior to the date of the effectiveness of the registration statement of which this prospectus forms a part. Any outstanding awards granted under the 2014 Plan will remain outstanding following the offering, subject to the terms of our 2014 Plan and applicable award agreements, until such awards are exercised or until they terminate or expire by their terms.

2019 equity incentive plan

We have adopted our 2019 Equity Incentive Plan, or the 2019 Plan, that will become effective on the date immediately prior to the date of the effectiveness of the registration of which this prospectus forms a part and will serve as the successor to our 2014 Plan. Our 2019 Plan authorizes the award of stock options, restricted stock awards, or RSAs, stock appreciation rights, or SARs, restricted stock units, or RSUs, performance awards, cash awards and stock bonus awards. We have initially reserved 2,200,000 shares of our common stock, plus any reserved shares not issued or subject to outstanding grants under the 2014 Plan on the effective date of the 2019 Plan, for issuance pursuant to awards granted under our 2019 Plan. The number of shares reserved for issuance under our 2019 Plan will increase automatically on January 1 of each of 2020 through 2029 by the number of shares equal to 4% of the aggregate number of outstanding shares of our common stock as of the immediately preceding December 31, or a lesser number as may be determined by our board of directors.

In addition, the following shares will again be available for issuance pursuant to awards granted under our 2019 Plan:

- shares subject to options or SARs granted under our 2019 Plan that cease to be subject to the option or SAR for any reason other than exercise of the option or SAR;
- shares subject to awards granted under our 2019 Plan that are subsequently forfeited or repurchased by us at the original issue price;
- shares subject to awards granted under our 2019 Plan that otherwise terminate without such shares being issued;
- shares subject to awards granted under our 2019 Plan that are surrendered, cancelled or exchanged for cash or a different award (or combination thereof);
- shares issuable upon the exercise of options or subject to other awards granted under our 2014 Plan that cease to be subject to such options or other awards, by forfeiture or otherwise, after the termination of the 2014 Plan;
- shares subject to awards granted under our 2014 Plan that are forfeited or repurchased by us at the original price after the termination of the 2014 Plan; and
- shares subject to awards under our 2014 Plan or our 2019 Plan that are used to pay the exercise price of an option or withheld to satisfy the tax withholding obligations related to any award.

Administration. Our 2019 Plan is expected to be administered by our compensation committee, or by our board of directors acting in place of our compensation committee. Subject to the terms and conditions of the

2019 Plan, the compensation committee will have the authority, among other things, to select the persons to whom awards may be granted, construe and interpret our 2019 Plan as well as to determine the terms of such awards and prescribe, amend and rescind the rules and regulations relating to the plan or any award granted thereunder. The 2019 Plan provides that the board or compensation committee may delegate its authority, including the authority to grant awards, to one or more executive officers to the extent permitted by applicable law, provided that awards granted to non-employee directors may only be determined by our board of directors.

Eligibility. Our 2019 Plan provides for the grant of awards to our employees, directors, consultants, independent contractors and advisors. No non-employee director may receive awards under our 2019 Plan that, when combined with cash compensation received for services as a non-employee director, exceed $500,000 in a calendar year or $1,000,000 in the calendar year of his or her initial services as a non-employee director with us.

Options. The 2019 Plan provides for the grant of both incentive stock options intended to qualify under Section 422 of the Code, and non-statutory stock options to purchase shares of our common stock at a stated exercise price. Incentive stock options may only be granted to employees, including officers and directors who are also employees. The exercise price of stock options granted under the 2019 Plan must be at least equal to the fair market value of our common stock on the date of grant. Incentive stock options granted to an individual who holds, directly or by attribution, more than ten percent of the total combined voting power of all classes of our capital stock must have an exercise price of at least 110% the fair market value of our common stock on the date of grant. Subject to stock splits, dividends, recapitalizations or similar events, no more than 22,000,000 shares may be issued pursuant to the exercise of incentive stock options granted under the 2019 Plan.

Options may vest based on service or achievement of performance conditions. Our compensation committee may provide for options to be exercised only as they vest or to be immediately exercisable, with any shares issued on exercise being subject to our right of repurchase that lapses as the shares vest. The maximum term of options granted under our 2019 Plan is ten years from the date of grant, except that the maximum permitted term of incentive stock options granted to an individual who holds, directly or by attribution, more than ten percent of the total combined voting power of all classes of our capital stock is five years from the date of grant.

Restricted stock awards. An RSA is an offer by us to sell shares of our common stock subject to restrictions, which may lapse based on the satisfaction of service or achievement of performance conditions. The price, if any, of an RSA will be determined by the compensation committee. Holders of RSAs, unlike holders of options, will have the right to vote and any dividends or stock distributions paid pursuant to RSAs will be accrued and paid when the restrictions on such shares lapse. Unless otherwise determined by the compensation committee at the time of award, vesting will cease on the date the participant no longer provides services to us and unvested shares may be forfeited to or repurchased by us.

Stock appreciation rights. A SAR provides for a payment, in cash or shares of our common stock (up to a specified maximum of shares, if determined by our compensation committee), to the holder based upon the difference between the fair market value of our common stock on the date of exercise and a predetermined exercise price, multiplied by the number of shares. The exercise price of a SAR must be at least equal to the fair market value of a share of our common stock on the date of grant. SARs may vest based on service or achievement of performance conditions, and may not have a term that is longer than ten years from the date of grant.

Restricted stock units. RSUs represent the right to receive shares of our common stock at a specified date in the future, and may be subject to vesting based on service or achievement of performance conditions. Payment of earned RSUs will be made as soon as practicable on a date determined at the time of grant, and may be

settled in cash, shares of our common stock or a combination of both. No RSU may have a term that is longer than ten years from the date of grant.

Performance awards. Performance awards granted to pursuant to the 2019 Plan may be in the form of a cash bonus, or an award of performance shares or performance units denominated in shares of our common stock that may be settled in cash, property or by issuance of those shares subject to the satisfaction or achievement of specified performance conditions.

Stock bonus awards. A stock bonus award provides for payment in the form of cash, shares of our common stock or a combination thereof, based on the fair market value of shares subject to such award as determined by our compensation committee. The awards may be granted as consideration for services already rendered, or at the discretion of the compensation committee, may be subject to vesting restrictions based on continued service or performance conditions.

Cash awards. A cash award is an award that is denominated in, or payable to an eligible participant solely in, cash.

Dividend equivalents rights. Dividend equivalent rights may be granted at the discretion of our compensation committee, and represent the right to receive the value of dividends, if any, paid by us in respect of the number of shares of our common stock underlying an award. Dividend equivalent rights will be subject to the same vesting or performance conditions as the underlying award and will be paid only at such time as the underlying award has become fully vested. Dividend equivalent rights may be settled in cash, shares or other property, or a combination of thereof as determined by the compensation committee.

Change of control. In the event of a “corporate transaction” (as defined in the 2019 Plan), awards may be assumed, converted, replaced, or substituted by the successor corporation, which assumption, conversion, replacement or substitution will be binding on all participants. In the event of a substitution, the successor corporation may substitute equivalent awards or provide substantially similar consideration to participants as was provided to stockholders (after taking into account the existing provisions of the awards). In the event such successor or acquiring corporation (if any) refuses to assume, convert, replace, or substitute awards, as provided above, pursuant to a corporate transaction, then notwithstanding any other provision in the 2019 Plan to the contrary, such awards shall have their vesting accelerate as to all shares or cash subject to such awards (and any applicable right of repurchase shall fully lapse) immediately prior to the corporate transaction and all such awards shall expire on such corporate transaction at such time and on such conditions as our board of directors determine. In addition, in the event such successor or acquiring corporation (if any) refuses to assume, convert, replace, or substitute awards, as provided above, pursuant to a corporate transaction, the committee will notify the participant in writing or electronically that such participant’s award will, if exercisable, be exercisable for a period of time determined by the committee in its sole discretion, and such award will terminate upon the expiration of such period. Awards need not all be treated in the same manner in a corporate transaction, and treatment may vary from award to award and/or from participant to participant.

Adjustment. In the event of a change in the number of outstanding shares of our common stock without consideration by reason of a stock dividend, extraordinary dividend or distribution, recapitalization, stock split, reverse stock split, subdivision, combination, consolidation reclassification, spin-off or similar change in our capital structure, appropriate proportional adjustments will be made to the number of shares reserved for issuance under our 2019 Plan; the exercise prices, number and class of shares subject to outstanding options or SARs; the number and class of shares subject to other outstanding awards; and any applicable maximum award limits with respect to incentive stock options.

Clawback; transferability. All awards will be subject to clawback or recoupment pursuant to any compensation clawback or recoupment policy adopted by our board of directors or required by law during the term of service

of the award holder, to the extent set forth in such policy or applicable agreement. Except in limited circumstances, awards granted under our 2019 Plan may generally not be transferred in any manner prior to vesting other than by will or by the laws of descent and distribution.

Amendment and termination. Our board of directors may amend our 2019 Plan at any time, subject to stockholder approval as may be required. Our 2019 Plan will terminate ten years from the date our board of directors adopts the plan, unless it is terminated earlier by our board of directors. No termination or amendment of the 2019 Plan may adversely affect any then-outstanding award without the consent of the affected participant, except as is necessary to comply with applicable laws.

2019 employee stock purchase plan

We have adopted a 2019 Employee Stock Purchase Plan that will become effective on the date of this prospectus and will enable eligible employees to purchase shares of our common stock at a discount beginning on a date determined by our board of directors or our compensation committee. Purchases will be accomplished through participation in discrete offering periods. We initially reserved 315,000 shares of our common stock for issuance under our 2019 Employee Stock Purchase Plan. The number of shares reserved for issuance under our 2019 Employee Stock Purchase Plan will increase automatically on January 1st of each of the first 10 calendar years following the first offering date by the number of shares equal to the lesser of 1% of the total outstanding shares of our common stock as of the immediately preceding December 31 or a lower amount determined by our board of directors. The aggregate number of shares issued over the term of our 2019 Employee Stock Purchase Plan will not exceed 3,150,000 shares of our common stock. Our 2019 Employee Stock Purchase Plan is intended to qualify as an employee stock purchase plan under Section 423 of the Code.

Our compensation committee will administer our 2019 Employee Stock Purchase Plan. While our employees generally are eligible to participate in our 2019 Employee Stock Purchase Plan, our compensation committee may in its discretion elect to exclude employees who work less than 20 hours per week or less than five months in a calendar year. In addition, employees who are 5% stockholders, or would become 5% stockholders as a result of their participation in our 2019 Employee Stock Purchase Plan, are ineligible to participate in our 2019 Employee Stock Purchase Plan. We may impose additional restrictions on eligibility within the limits permitted by the Code. Under our 2019 Employee Stock Purchase Plan, eligible employees will be able to acquire shares of our common stock by accumulating funds through payroll deductions.

When a first offering period commences, our employees who meet the eligibility requirements for participation in that offering period will be eligible to enroll. For subsequent offering periods, new participants will be required to enroll in a timely manner. Once an employee is enrolled, participation will be automatic in subsequent offering periods. Each offering period will be determined by our compensation committee. An employee's participation automatically ends upon termination of employment for any reason.

The first offering period will begin on a future date to be designated by our board of directors or compensation committee. Each subsequent offering period will be designated by our compensation committee, but will in no event be longer than 27 months.

No participant will have the right to purchase our shares in an amount, when aggregated with purchase rights under all our employee stock purchase plans that are also in effect in the same calendar years, that has a fair market value of more than $25,000, determined as of the first day of the applicable offering period, for each calendar year in which that right is outstanding. In addition, no participant will be permitted to purchase more than 2,500 shares during any one purchase period or such greater or lesser amount determined by our compensation committee. The purchase price for shares of our common stock purchased under our 2019 Employee Stock Purchase Plan will be 85% of the lesser of the fair market value of our common stock on (1) the

first trading day of the applicable offering period or (2) the last trading day of each purchase period in the applicable offering period.

If we experience a corporate transaction (as defined in the 2019 Employee Stock Purchase Plan), each outstanding right to purchase shares under our 2019 Employee Stock Purchase Plan will be assumed or an equivalent option substituted by the successor corporation. In the event that the successor corporation refuses to assume or substitute for the outstanding purchase rights, any offering period that commenced prior to the closing of the proposed corporate transaction will be shortened and terminated on a new purchase date. The new purchase date will occur on or prior to the consummation of the corporate transaction and our 2019 Employee Stock Purchase Plan will then terminate on the consummation of the corporate transaction.

We will also have the right to amend or terminate our 2019 Employee Stock Purchase Plan at any time. Our 2019 Employee Stock Purchase Plan will terminate on the tenth anniversary of the last day of the first purchase period, unless it is terminated earlier by our board of directors.

401(k) plan

We sponsor a retirement savings plan, or 401(k) plan, that is intended to qualify for favorable tax treatment under Section 401(a) of the Code, and contains a cash or deferred feature that is intended to meet the requirements of Section 401(k) of the Code. U.S. employees who have attained at least 21 years of age are generally eligible to participate in the 401(k) plan following two months of service, subject to certain criteria. Participants may make pre-tax and certain after-tax (Roth) salary deferral contributions to the plan from their eligible earnings up to the statutorily prescribed annual limit under the Code. Participants who are 50 years of age or older may contribute additional amounts based on the statutory limits for catch-up contributions. Participant contributions are held in trust as required by law. No minimum benefit is provided under the plan. An employee's interest in his or her salary deferral contributions is 100% vested when contributed. We have the ability to make discretionary matching and profit share contributions under the plan but have not done so to date. Any such discretionary employer contributions would vest in equal, annual installments over 4 years and may be subject to other eligibility requirements.

Other benefits

Our named executive officers are eligible to participate in our employee benefit plans on the same basis as our other employees, including our health and welfare plans.

Limitations on liability and indemnification matters

Our restated certificate of incorporation that will become effective in connection with the completion of this offering contains provisions that limit the liability of our directors for monetary damages to the fullest extent permitted by the Delaware General Corporation Law, or DGCL. Consequently, our directors will not be personally liable to us or our stockholders for monetary damages for any breach of fiduciary duties as directors, except liability for:

- any breach of the director's duty of loyalty to us or our stockholders;
- any act or omission not in good faith or that involves intentional misconduct or a knowing violation of law;
- unlawful payments of dividends or unlawful stock repurchases or redemptions as provided in Section 174 of the DGCL; or
- any transaction from which the director derived an improper personal benefit.

Our restated certificate of incorporation and our restated bylaws that will become effective in connection with the completion of this offering require us to indemnify our directors and officers to the maximum extent not prohibited by the DGCL and allow us to indemnify other employees and agents as set forth in the DGCL.

We have entered, and intend to continue to enter, into separate indemnification agreements with our directors, officers and certain of our key employees, in addition to the indemnification provided for in our restated certificate of incorporation and restated bylaws. These agreements, among other things, require us to indemnify our directors, officers and key employees for certain expenses, including attorneys' fees, judgments, penalties, fines and settlement amounts actually incurred by these individuals in any action or proceeding arising out of their service to us or any of our subsidiaries or any other company or enterprise to which these individuals provide services at our request. Subject to certain limitations, our indemnification agreements also require us to advance expenses incurred by our directors, officers and key employees for the defense of any action for which indemnification is required or permitted.

We believe that these indemnification provisions and agreements are necessary to attract and retain qualified directors, officers and key employees. We also maintain directors' and officers' liability insurance.

The limitation of liability and indemnification provisions in our restated certificate of incorporation and restated bylaws may discourage stockholders from bringing a lawsuit against our directors and officers for breach of their fiduciary duty. They may also reduce the likelihood of derivative litigation against our directors and officers, even though an action, if successful, might benefit us and other stockholders. Further, a stockholder's investment may be adversely affected to the extent that we pay the costs of settlement and damage awards against directors and officers as required by these indemnification provisions.

At present, there is no pending litigation or proceeding involving any of our directors or executive officers as to which indemnification is required or permitted, and we are not aware of any threatened litigation or proceeding that may result in a claim for indemnification.

Insofar as indemnification for liabilities arising under the Securities Act of 1933, as amended, or Securities Act, may be permitted to directors, executive officers or persons controlling us, we have been informed that, in the opinion of the SEC, such indemnification is against public policy as expressed in the Securities Act and is therefore unenforceable.

Certain relationships and related party transactions

In addition to the compensation arrangements, including any employment, termination of employment and change in control arrangements, with our directors and executive officers, including those discussed in the sections entitled “Management” and “Executive compensation,” the following is a description of each transaction since January 1, 2016 and each currently proposed transaction in which:

- we have been or are to be a participant;
- the amounts involved exceeded or will exceed $120,000; and
- any of our directors, executive officers or holders of more than 5% of our capital stock, or an affiliate or immediate family member of the foregoing persons, had or will have a direct or indirect material interest.

Other than as described below, there have not been, nor are there any currently proposed, transactions or series of similar transactions to which we have been or will be a party other than compensation arrangements, which are described where required under the section entitled “Executive compensation.”

Equity financings

Series A

In July 15, 2015, we sold an aggregate of 2,458,564 shares of our Series A convertible preferred stock at a purchase price of $2.38 per share for an aggregate purchase price of approximately $5.8 million. In July 2016, we sold an aggregate of 1,260,802 additional shares of our Series A convertible preferred stock at a purchase price of $2.38 per share for an aggregate purchase price of approximately $3.0 million. In February 2017, we sold an aggregate of 1,260,802 additional shares of our Series A convertible preferred stock at a purchase price of $2.38 per share for an aggregate purchase price of approximately $3.0 million.

Simple Agreement for Future Equity

In October 2017, we issued, to Apple Tree Partners IV, L.P., rights to purchase certain shares of our capital stock for an aggregate purchase price of $3,000,000, or Purchase Amount, pursuant to a Simple Agreement for Future Equity, or SAFE. The entire Purchase Amount and all other obligations under the SAFE converted into 788,042 shares of our Series A-2 convertible preferred stock, which is described below.

Series A-2

In January 2018, we sold an aggregate of 4,071,554 shares of our Series A-2 convertible preferred stock at a purchase price of $3.81 per share for an aggregate purchase price of approximately $15.5 million. In September 2018, we sold an aggregate of 3,546,192 additional shares of our Series A-2 convertible preferred stock at a purchase price of $3.81 per share for an aggregate purchase price of approximately $13.5 million.

Series B

In October 2018, we sold an aggregate of 10,079,671 shares of our Series B convertible preferred stock at a purchase price of $8.93 per share for an aggregate purchase price of approximately $90 million.

The following table summarizes the Series A, Series A-2 and Series B convertible preferred stock purchased by members of our board of directors or their affiliates and holders of more than five percent of our outstanding capital stock:

Name of stockholder	Series	Shares of convertible preferred stock	Total purchase price ($)
Apple Tree Partners, IV, L.P.(1)	A	4,980,170	11,849,999.57
Apple Tree Partners, IV, L.P.(1)	A-2	7,617,746	28,999,999.50
Apple Tree Partners, IV, L.P.(1)	B	2,799,910	24,999,999.18
RTW Master Fund(2)	B	2,430,157	21,698,526.75
RTW Innovation Master Fund, Ltd.(2)	B	369,753	3,301,472.43

1. Apple Tree Partners, IV, L.P., or ATP IV, holds more than five percent of our outstanding capital stock. Seth L. Harrison, M.D., a member of our board of directors, is the founder and managing partner of ATP IV and Samuel W. Hall, Ph.D., a member of our board of directors, is a principal at ATP IV.

2. Entities associated with RTW Investments, or RTW, hold more than 5% of our outstanding capital stock.

Amended and restated investors' rights agreement

We have entered into a second amended and restated investors' rights agreement, or the IRA, dated October 22, 2018, with certain holders of our convertible preferred stock, including entities with which certain of our directors are affiliated. Additionally, the IRA provides for a participation right to affiliates of RTW Master Fund and Apple Tree Partners IV, L.P., holders of more than five percent of our common stock, to purchase a specified percentage of shares of common stock in this offering at the public offering price. The IRA further provides that, under certain circumstances in which such entities are unable to participate in this offering, we are required to offer them shares of our common stock through a separate private placement to be concurrent with this offering. Under the IRA, these stockholders are also entitled to rights with respect to the registration of their shares following this offering under the Securities Act of 1933, as amended. Finally, the IRA provides that for so long as any of Apple Tree Partners IV, L.P., RTW Master Fund or RTW Innovation Master Fund, Ltd., or any of their respective affiliates, holds any shares of our preferred stock or common stock issued upon conversion thereof, such investor or investors shall have the right to attend our board meetings in a nonvoting observer capacity. For a description of these registration rights, see the section entitled "Description of capital stock—Registration rights."

Equity grants to executive officers and directors

We have granted stock options to our executive officers and certain directors, as more fully described in the sections entitled "Executive compensation" and "Management—Non-employee director compensation," respectively.

Director and executive officer compensation

Please see the sections entitled "Management—Non-employee director compensation" and "Executive compensation" for information regarding the compensation of our directors and executive officers.

Employment agreements

We have entered into employment agreements with our executive officers. For more information regarding these agreements, see the section entitled "Executive compensation—Employment agreements."

Consulting agreement

In October 2014, we entered into a consulting agreement with Adrian R. Krainer, who is also an employee of Cold Spring Harbor Laboratory, to provide consulting services related to scientific research related to the development of antisense-based drugs, therapies, diagnostic and research tools, products, services and intellectual property. We made payments of $100,000 during each of the years ended December 31, 2018, 2017 and 2016 for such consulting services. The initial term of this agreement was five years and may be extended by the mutual consent of us and Professor Krainer.

Indemnification agreements

In connection with this offering, we intend to enter into new indemnification agreements with each of our directors and executive officers. The indemnification agreements, our restated certificate of incorporation and our restated bylaws will require us to indemnify our directors to the fullest extent not prohibited by Delaware law. Subject to certain limitations, our restated bylaws also require us to advance expenses incurred by our directors and officers. For more information regarding these agreements, see the section entitled “Executive compensation—Limitations on liability and indemnification matters” for information on our indemnification arrangements with our directors and executive officers.

Policies and procedures for related party transactions

In connection with this offering, we intend to adopt a written related person transactions policy that provides that our executive officers, directors, nominees for election as a director, beneficial owners of more than 5% of our common stock, and any members of the immediate family of and any entity affiliated with any of the foregoing persons, are not permitted to enter into a material related person transaction with us without the review and approval of our audit committee, or a committee composed solely of independent directors in the event it is inappropriate for our audit committee to review such transaction due to a conflict of interest. We expect the policy to provide that any request for us to enter into a transaction with an executive officer, director, nominee for election as a director, beneficial owner of more than 5% of our common stock or with any of their immediate family members or affiliates in which the amount involved exceeds $120,000 will be presented to our audit committee (or the committee composed solely of independent directors, if applicable) for review, consideration and approval. In approving or rejecting any such proposal, we expect that our audit committee (or the committee composed solely of independent directors, if applicable) will consider the relevant facts and circumstances available and deemed relevant to the audit committee (or the committee composed solely of independent directors, if applicable), including, but not limited to, whether the transaction is on terms no less favorable than terms generally available to an unaffiliated third party under the same or similar circumstances and the extent of the related person’s interest in the transaction.

Principal stockholders

The following table and accompanying footnotes set forth certain information with respect to the beneficial ownership of our common stock at May 31, 2019, and as adjusted to reflect the shares of common stock to be issued and sold in this offering, for:

- each of our directors;
- each of our named executive officers;
- all of our current directors and executive officers as a group; and
- each person, or group of affiliated persons, who beneficially owned more than five percent of our outstanding shares of common stock.

We have determined beneficial ownership in accordance with the rules of the SEC, and the information is not necessarily indicative of beneficial ownership for any other purpose. Except as indicated by the footnotes below, we believe, based on information furnished to us, that the persons and entities named in the table below have sole voting and sole investment power with respect to all shares of common stock that they beneficially owned, subject to applicable community property laws.

Beneficial ownership prior to this offering is based on 23,569,808 shares of common stock outstanding as of May 31, 2019, assuming the automatic conversion of all outstanding shares of our convertible preferred stock into common stock in connection with this offering. Beneficial ownership after this offering is based on 30,269,808 shares of common stock outstanding, assuming (i) the automatic conversion of all outstanding shares of our convertible preferred stock into common stock as described above and (ii) the issuance of 6,700,000 shares of common stock in this offering. In computing the number of shares of common stock beneficially owned by a person and the percentage ownership of that person, we deemed to be outstanding all shares of common stock subject to options held by that person or entity that are currently exercisable or that will become exercisable within 60 days of May 31, 2019. We did not deem these shares outstanding, however, for the purpose of computing the percentage ownership of any other person.

Unless otherwise indicated, the address of each beneficial owner listed in the table below is c/o Stoke Therapeutics, Inc., 45 Wiggins Avenue, Bedford, Massachusetts, 01730.

	Beneficial ownership prior to this offering		Beneficial ownership after this offering	
Name of beneficial owner	**Number**	**Percent**	**Number**	**Percent**
Directors and named executive officers:				
Edward M. Kaye, M.D.(1)	393,108	1.6%	393,108	1.3%
Huw M. Nash, Ph.D.(2)	332,104	1.4	332,104	1.1
Barry S. Ticho, M.D., Ph.D., FACC(3)	119,098	*	119,098	*
Jennifer C. Burstein, CPA	—	—	—	—
Seth L. Harrison, M.D.(4)	15,397,824	65.3	15,397,824	50.9
Samuel W. Hall, Ph.D.	—	—	—	—
Adrian R. Krainer, Ph.D.(5)	413,292	1.8	413,292	1.4
Arthur A. Levin, Ph.D.(6)	27,459	*	27,459	*
Arthur O. Tzianabos, Ph.D.(7)	12,862	*	12,862	*
All executive officers and directors as a group (11 persons)(8)	16,771,126	68.4	16,771,126	53.7
Other 5% stockholders:				
Apple Tree Partners IV, L.P.(4)	15,397,824	65.3	15,397,824	50.9
Entities affiliated with RTW Investments, L.P.(9)	2,799,910	11.9	2,799,910	9.3

* Represents beneficial ownership of less than one percent.

(1) Represents 393,108 shares underlying options to purchase common stock that are exercisable within 60 days of May 31, 2019.

(2) Represents 332,104 shares underlying options to purchase common stock that are exercisable within 60 days of May 31, 2019.

(3) Represents 119,098 shares underlying options to purchase common stock that are exercisable within 60 days of May 31, 2019.

(4) Represents 15,397,824 shares of common stock held by Apple Tree Partners IV, L.P., or ATP IV. ATP III GP, Ltd., or ATP III, is the sole general partner of ATP IV. Seth L. Harrison, M.D., a member of our board of directors, is the sole director of ATP III and may be deemed to have sole voting and dispositive power over the shares held by ATP IV. The address of ATP IV is 230 Park Avenue, Suite 2800, New York, New York 10169.

(5) Represents (i) 404,718 shares of common stock and (ii) 8,574 shares underlying options to purchase common stock that are exercisable within 60 days of May 31, 2019.

(6) Represents (i) 17,979 shares of common stock held by Arthur A. Levin, Trustee, Butler-Levin Revocable Trust and (ii) 9,480 shares underlying options to purchase common stock that are exercisable within 60 days of May 31, 2019.

(7) Represents 12,862 shares underlying options to purchase common stock that are exercisable within 60 days of May 31, 2019.

(8) Represents (i) 15,820,521 shares of common stock and (ii) 950,605 shares underlying options to purchase common stock that are exercisable within 60 days of May 31, 2019.

(9) Represents (i) 2,430,157 shares of common stock held by RTW Master Fund, Ltd., or RTW Master Fund, and (ii) 369,753 shares of common stock held by RTW Innovation Master Fund, Ltd., or RTW Innovation Fund. RTW Investments, LP, or RTW Investments, is the manager of RTW Master Fund and RTW Innovation Fund. Roderick Wong, M.D. is the managing partner and chief investment officer of RTW Investments and has sole voting and investment control over the shares held by each of RTW Master Fund and RTW Innovation Fund. The address of RTW Investments is 412 West 15th Street, Floor 9, New York, New York 10011.

Description of capital stock

The following description summarizes the most important terms of our capital stock, as they will be in effect following this offering. Because it is only a summary, it does not contain all the information that may be important to you. We expect to adopt a restated certificate of incorporation and restated bylaws that will become effective upon the completion of this offering, and this description summarizes provisions that are expected to be included in these documents. For a complete description, you should refer to our restated certificate of incorporation and restated bylaws, which are included as exhibits to the registration statement of which this prospectus forms a part, and to the applicable provisions of Delaware law.

Upon the completion of this offering, our authorized capital stock will consist of 300,000,000 shares of common stock, $0.0001 par value per share, and 10,000,000 shares of undesignated preferred stock, $0.0001 par value per share.

Pursuant to the provisions of our current certificate of incorporation all of the outstanding convertible preferred stock will automatically convert into common stock in connection with the completion of this offering. Our Series A convertible preferred stock will convert at a ratio of 1:1, our Series A-2 convertible preferred stock will convert at a ratio of 1:1, and our Series B convertible preferred stock will convert at a ratio of 1:1. Assuming the effectiveness of this conversion as of March 31, 2019, there were 23,569,808 shares of our common stock issued, held by approximately 24 stockholders of record, and no shares of our convertible preferred stock outstanding. Our board of directors is authorized, without stockholder approval, to issue additional shares of our capital stock.

Common stock

Dividend rights

Subject to preferences that may apply to any shares of preferred stock outstanding at the time, the holders of our common stock are entitled to receive dividends out of funds legally available if our board of directors, in its discretion, determines to issue dividends and then only at the times and in the amounts that our board of directors may determine. See the section entitled “Dividend policy.”

Voting rights

Holders of our common stock are entitled to one vote for each share held on all matters submitted to a vote of stockholders. We have not provided for cumulative voting for the election of directors in our restated certificate of incorporation, which means that holders of a majority of the shares of our common stock will be able to elect all of our directors. Our restated certificate of incorporation will establish a classified board of directors, to be divided into three classes with staggered three-year terms. Only one class of directors will be elected at each annual meeting of our stockholders, with the other classes continuing for the remainder of their respective three-year terms.

No preemptive or similar rights

Our common stock is not entitled to preemptive rights, and is not subject to conversion, redemption or sinking fund provisions.

Right to receive liquidation distributions

Upon our liquidation, dissolution or winding-up, the assets legally available for distribution to our stockholders would be distributable ratably among the holders of our common stock and any participating preferred stock outstanding at that time, subject to prior satisfaction of all outstanding debt and liabilities and the preferential rights of and the payment of liquidation preferences, if any, on any outstanding shares of preferred stock.

Preferred stock

Immediately prior to the completion of this offering, each outstanding share of preferred stock will be converted into common stock at a ratio of 1:1.

Following the completion of this offering, our board of directors will be authorized, subject to limitations prescribed by Delaware law, to issue preferred stock in one or more series, to establish from time to time the number of shares to be included in each series and to fix the designation, powers, preferences and rights of the shares of each series and any of their qualifications, limitations or restrictions, in each case without further vote or action by our stockholders. Our board of directors will also be able to increase or decrease the number of shares of any series of preferred stock, but not below the number of shares of that series then outstanding, without any further vote or action by our stockholders. Our board of directors may authorize the issuance of preferred stock with voting or conversion rights that could adversely affect the voting power or other rights of the holders of our common stock. The issuance of preferred stock, while providing flexibility in connection with possible acquisitions and other corporate purposes, could, among other things, have the effect of delaying, deferring or preventing a change in control of our company and might adversely affect the market price of our common stock and the voting and other rights of the holders of our common stock. We have no current plan to issue any shares of preferred stock.

Stock options

As of March 31, 2019, we had outstanding options to purchase an aggregate 4,034,649 shares of our common stock, with a weighted-average exercise price of $1.64.

Registration rights

Pursuant to the terms of our amended and restated investors' rights agreement, immediately following this offering, the holders of 22,677,585 shares of our common stock will be entitled to rights with respect to the registration of these shares under the Securities Act of 1933, as amended, or the Securities Act, as described below. We refer to these shares collectively as registrable securities.

Form S-1 registration rights

Beginning 180 days after the completion of this offering, the holders of at least a majority of the then-outstanding registrable securities may make a request to us for the registration under the Securities Act of registrable securities if the aggregate price to the public of the shares offered is at least $10.0 million. Within ten (10) days following such request, we are obligated to provide notice of such request to all stockholders, other than the initiating holders, to file a registration statement under the Securities Act covering all registrable securities that the initiating holders requested to be registered and any additional registrable securities requested to be included in such registration by any other holders. We are only required to file two registration statements that are declared effective upon exercise of these demand registration rights. We may postpone taking action with respect to such filing not more than once during any 12-month period for a total period of not more than 120 days, if after receiving a request for registration, we furnish to the holders requesting such registration a certificate signed by our Chief Executive Officer stating that, in the good faith judgment of our board of directors, it would be materially detrimental to us and our stockholders for such registration statement to be effected at such time.

The underwriters of any underwritten offering will have the right to limit the number of shares registered by these holders if they determine that marketing factors require limitation, in which case the number of shares to be registered will be apportioned, in proportion (as nearly as practicable), to the number of registrable securities owned by each holder or in such other proportion as shall mutually be agreed to by all such selling

Holders. However, the number of shares to be registered by these holders cannot be reduced unless all other securities are first entirely excluded from the underwriting.

Form S-3 registration rights

Any holder or group of holders of at least 20% of then-outstanding registrable securities can request that we register all or part of their shares on Form S-3 if we are eligible to file a registration statement on Form S-3 and if the aggregate price to the public of the shares offered is at least $1.0 million. The stockholders may only require us to effect two registration statements on Form S-3 in a 12-month period. We may postpone taking action with respect to such filing not more than once during any 12-month period for a total period of not more than 120 days, if after receiving a request for registration, we furnish to the holders requesting such registration a certificate signed by our Chief Executive Officer stating that, in the good faith judgment of our board of directors, it would be materially detrimental to us and our stockholders for such registration statement to be effected at such time.

The underwriters of any underwritten offering will have the right to limit the number of shares registered by these holders if they determine that marketing factors require limitation, in which case the number of shares to be registered will be apportioned, in proportion (as nearly as practicable), to the number of registrable securities owned by each holder or in such other proportion as shall mutually be agreed to by all such selling Holders. However, the number of shares to be registered by these holders cannot be reduced unless all other securities are first entirely excluded from the underwriting.

Piggyback registration rights

If we register any of our securities for public sale, holders of then-outstanding registrable securities or their permitted transferees will have the right to include their registrable securities in the registration statement. However, this right does not apply to a registration relating to employee benefit plans, a registration relating to a corporate reorganization, a registration on a form that does not include substantially the same information as would be required to be included in a registration statement covering the sale of registrable securities or a registration in which the only common stock being registered is common stock issuable upon conversion of debt securities that are being registered.

The underwriters of any underwritten offering will have the right to limit the number of shares registered by these holders if they determine that marketing factors require limitation, in which case the number of shares to be registered will be apportioned to the selling holders, in proportion (as nearly as practicable), to the number of registrable securities owned by each selling holder or in such other proportion as shall mutually be agreed to by all such selling Holders. However, the number of shares to be registered by these holders cannot be reduced (i) unless all other securities (other than securities to be sold by us) are first entirely excluded from the offering, (ii) below 25% of the total number of securities included in such offering, unless such offering is the initial public offering, in which case the selling holders may be excluded further if the underwriters make the determination for a limitation and no other stockholder's securities are included in such offering.

Expenses of registration rights

We generally will pay all expenses, other than underwriting discounts and commissions.

Expiration of registration rights

The registration rights described above will expire, with respect to any particular holder of these rights, on the earlier of the fifth anniversary of this offering or with respect to each holder, such time following this offering as all registrable securities of such holder may be sold within a three-month period pursuant to Rule 144.

Anti-takeover provisions

The provisions of Delaware General Corporation Law, or DGCL, our restated certificate of incorporation and our restated bylaws, as we expect they will be in effect upon the completion of this offering, could have the effect of delaying, deferring or discouraging another person from acquiring control of our company. These provisions, which are summarized below, may have the effect of discouraging takeover bids. They are also designed, in part, to encourage persons seeking to acquire control of us to negotiate first with our board of directors. We believe that the benefits of increased protection of our potential ability to negotiate with an unfriendly or unsolicited acquirer outweigh the disadvantages of discouraging a proposal to acquire us because negotiation of these proposals could result in an improvement of their terms.

Delaware law

We are subject to the provisions of Section 203 of the DGCL regulating corporate takeovers. In general, Section 203 prohibits a publicly held Delaware corporation from engaging in a "business combination" with an "interested stockholder" for a period of three years following the date on which the person became an interested stockholder unless:

- prior to the date of the transaction, the board of directors of the corporation approved either the business combination or the transaction which resulted in the stockholder becoming an interested stockholder;
- the interested stockholder owned at least 85% of the voting stock of the corporation outstanding at the time the transaction commenced, excluding for purposes of determining the voting stock outstanding, but not the outstanding voting stock owned by the interested stockholder, (i) shares owned by persons who are directors and also officers and (ii) shares owned by employee stock plans in which employee participants do not have the right to determine confidentially whether shares held subject to the plan will be tendered in a tender or exchange offer; or
- at or subsequent to the date of the transaction, the business combination is approved by the board of directors of the corporation and authorized at an annual or special meeting of stockholders, and not by written consent, by the affirmative vote of at least 66.67% of the outstanding voting stock that is not owned by the interested stockholder.

Generally, a business combination includes a merger, asset or stock sale, or other transaction or series of transactions together resulting in a financial benefit to the interested stockholder. An interested stockholder is a person who, together with affiliates and associates, owns or, within three years prior to the determination of interested stockholder status, did own 15% or more of a corporation's outstanding voting stock. We expect the existence of this provision to have an anti-takeover effect with respect to transactions our board of directors does not approve in advance. We also anticipate that Section 203 may also discourage attempts that might result in a premium over the market price for the shares of common stock held by stockholders.

Restated certificate of incorporation and restated bylaw provisions

Our restated certificate of incorporation and our restated bylaws, as we expect they will be in effect upon the completion of this offering, include a number of provisions that could deter hostile takeovers or delay or prevent changes in control of our company, including the following:

- *Board of directors vacancies.* Our restated certificate of incorporation and restated bylaws will authorize only our board of directors to fill vacant directorships, including newly created seats. In addition, the number of directors constituting our board of directors is permitted to be set only by a resolution adopted by a majority vote of our entire board of directors. These provisions would prevent a stockholder from increasing the size of our board of directors and then gaining control of our board of directors by filling the resulting

vacancies with its own nominees. This makes it more difficult to change the composition of our board of directors but promotes continuity of management.

- *Classified board.* Our restated certificate of incorporation and restated bylaws will provide that our board of directors is classified into three classes of directors, each with staggered three-year terms. A third party may be discouraged from making a tender offer or otherwise attempting to obtain control of us as it is more difficult and time consuming for stockholders to replace a majority of the directors on a classified board of directors. See the section entitled “Management—Board composition.”
- *Stockholder action; special meetings of stockholders.* Our restated certificate of incorporation will provide that our stockholders may not take action by written consent, but may only take action at annual or special meetings of our stockholders. As a result, a holder controlling a majority of our capital stock would not be able to amend our restated bylaws or remove directors without holding a meeting of our stockholders called in accordance with our restated bylaws. Further, our restated bylaws will provide that special meetings of our stockholders may be called only by a majority of our board of directors, the chairman of our board of directors, our Chief Executive Officer or our President, thus prohibiting a stockholder from calling a special meeting. These provisions might delay the ability of our stockholders to force consideration of a proposal or for stockholders controlling a majority of our capital stock to take any action, including the removal of directors.
- *Advance notice requirements for stockholder proposals and director nominations.* Our restated bylaws will provide advance notice procedures for stockholders seeking to bring business before our annual meeting of stockholders or to nominate candidates for election as directors at our annual meeting of stockholders. Our restated bylaws also will specify certain requirements regarding the form and content of a stockholder’s notice. These provisions might preclude our stockholders from bringing matters before our annual meeting of stockholders or from making nominations for directors at our annual meeting of stockholders if the proper procedures are not followed. We expect that these provisions might also discourage or deter a potential acquirer from conducting a solicitation of proxies to elect the acquirer’s own slate of directors or otherwise attempting to obtain control of our company.
- *No cumulative voting.* The DGCL provides that stockholders are not entitled to the right to cumulate votes in the election of directors unless a corporation’s certificate of incorporation provides otherwise. Our restated certificate of incorporation and restated bylaws will not provide for cumulative voting.
- *Directors removed only for cause.* Our restated certificate of incorporation will provide that stockholders may remove directors only for cause and only by the affirmative vote of the holders of at least two-thirds of our outstanding common stock.
- *Amendment of charter provisions.* Any amendment of the above expected provisions in our restated certificate of incorporation would require approval by holders of at least two-thirds of our outstanding common stock.
- *Issuance of undesignated preferred stock.* Our board of directors has the authority, without further action by the stockholders, to issue up to 10,000,000 shares of undesignated preferred stock with rights and preferences, including voting rights, designated from time to time by our board of directors. The existence of authorized but unissued shares of preferred stock would enable our board of directors to render more difficult or to discourage an attempt to obtain control of us by merger, tender offer, proxy contest or other means.
- *Choice of forum.* Our restated certificate of incorporation will provide that, to the fullest extent permitted by law, the Court of Chancery of the State of Delaware will be the exclusive forum for any derivative action or

proceeding brought on our behalf; any action asserting a breach of fiduciary duty; any action asserting a claim against us arising pursuant to the DGCL, our restated certificate of incorporation or our restated bylaws; or any action asserting a claim against us that is governed by the internal affairs doctrine. The enforceability of similar choice of forum provisions in other companies' certificates of incorporation has been challenged in legal proceedings, and it is possible that a court could find these types of provisions to be inapplicable or unenforceable. This exclusive forum provision does not apply to suits brought to enforce a duty or liability created by the Exchange Act. It could apply, however, to a suit that falls within one or more of the categories enumerated in the exclusive forum provision and asserts claims under the Securities Act, inasmuch as Section 22 of the Securities Act creates concurrent jurisdiction for federal and state courts over all suits brought to enforce any duty or liability created by the Securities Act or the rules and regulations thereunder. There is uncertainty as to whether a court would enforce such provision with respect to claims under the Securities Act, and our stockholders will not be deemed to have waived our compliance with the federal securities laws and the rules and regulations thereunder.

- *Board Observer Rights.* Finally, the IRA provides that for so long as any of Apple Tree Partners IV, L.P., RTW Master Fund or RTW Innovation Master Fund, Ltd., or any of their respective affiliates, holds any shares of our preferred stock or common stock issued upon conversion thereof, such investor or investors shall have the right to attend our board meetings in a nonvoting observer capacity.

Transfer agent and registrar

Upon the completion of this offering, the transfer agent and registrar for our common stock will be American Stock Transfer & Trust Company, LLC. The transfer agent's address is 6201 15th Avenue, Brooklyn, New York 11219.

Nasdaq Global Market listing

We have applied to list our common stock on the Nasdaq Global Market under the symbol "STOK."

Shares eligible for future sale

Prior to this offering, there has been no public market for our common stock, and we cannot predict the effect, if any, that market sales of shares of our common stock or the availability of shares of our common stock for sale will have on the market price of our common stock prevailing from time to time. Nevertheless, sales of substantial amounts of our common stock, including shares issued upon exercise of outstanding options, in the public market following this offering could adversely affect market prices prevailing from time to time and could impair our ability to raise capital through the sale of our equity securities.

Upon the completion of this offering, we will have a total of 30,269,808 shares of our common stock outstanding, assuming (i) the automatic conversion of all outstanding shares of our convertible preferred stock into an aggregate of 22,677,585 shares of our common stock and (ii) the issuance of 6,700,000 shares of common stock in this offering. Of these outstanding shares, all of the shares of common stock sold in this offering will be freely tradable, except that any shares purchased in this offering by our affiliates, as that term is defined in Rule 144 under the Securities Act of 1933, as amended, or the Securities Act, can only be sold in compliance with the Rule 144 limitations described below or in compliance with the lock-up agreements.

The remaining outstanding shares of our common stock will be deemed “restricted securities” as defined in Rule 144. Restricted securities may be sold in the public market only if they are registered under the Securities Act or if they qualify for an exemption from registration under Rule 144 or Rule 701 promulgated under the Securities Act, which rules are summarized below. In addition, substantially all of our security holders have, or will have, entered into market standoff agreements with us or lock-up agreements with the underwriters under which they have agreed, subject to specific exceptions, not to sell any of our stock for at least 180 days following the date of this prospectus, as described below. As a result of these agreements and the provisions of our amended and restated investors’ rights agreement described above under the section entitled “Description of capital stock—Registration rights,” subject to the provisions of Rule 144 or Rule 701, shares will be available for sale in the public market as follows:

- beginning on the date of this prospectus, all of the shares sold in this offering will be immediately available for sale in the public market; and
- beginning 181 days after the date of this prospectus, 23,569,808 additional shares will become eligible for sale in the public market, of which 16,771,126 shares will be held by affiliates and subject to the volume and other restrictions of Rule 144, as described below.

Lock-up/market standoff agreements

All of our directors, officers and security holders are, or will be, subject to lock-up agreements or market standoff provisions that prohibit them from offering for sale, selling, contracting to sell, granting any option for the sale of, transferring or otherwise disposing of any shares of our common stock, options to acquire shares of our common stock or any security or instrument related to our common stock, or entering into any swap, hedge or other arrangement that transfers any of the economic consequences of ownership of our common stock, for a period of 180 days following the date of this prospectus without the prior written consent of J.P. Morgan Securities LLC, subject to certain exceptions. See the section entitled “Underwriting.”

Rule 144

In general, under Rule 144 as currently in effect, once we have been subject to public company reporting requirements for at least 90 days, a person who is not deemed to have been one of our affiliates for purposes of the Securities Act at any time during the three months preceding a sale and who has beneficially owned the

shares proposed to be sold for at least six months, including the holding period of any prior owner other than our affiliates, is entitled to sell those shares without complying with the manner of sale, volume limitation or notice provisions of Rule 144, subject to compliance with the public information requirements of Rule 144. If such a person has beneficially owned the shares proposed to be sold for at least one year, including the holding period of any prior owner other than our affiliates, then that person would be entitled to sell those shares without complying with any of the requirements of Rule 144.

In general, under Rule 144, as currently in effect, our affiliates or persons selling shares on behalf of our affiliates are entitled to sell upon expiration of the lock-up and market standoff agreements described above, within any three-month period, a number of shares that does not exceed the greater of:

- 1% of the number of shares of our common stock then outstanding, which will equal approximately 302,698 shares immediately after this offering; or
- the average reported weekly trading volume of our common stock during the four calendar weeks preceding the filing of a notice on Form 144 with respect to that sale.

Sales under Rule 144 by our affiliates or persons selling shares on behalf of our affiliates are also subject to certain manner of sale provisions and notice requirements and to the availability of current public information about us.

Rule 701

Rule 701 generally allows a stockholder who purchased shares of our common stock pursuant to a written compensatory plan or contract and who is not deemed to have been an affiliate of our company during the immediately preceding three months to sell these shares in reliance upon Rule 144, but without being required to comply with the public information, holding period, volume limitation or notice provisions of Rule 144. Rule 701 also permits affiliates of our company to sell their Rule 701 shares under Rule 144 without complying with the holding period requirements of Rule 144. All holders of Rule 701 shares, however, are required by that rule to wait until 90 days after the date of this prospectus before selling those shares pursuant to Rule 701 and are subject to the lock-up and market standoff agreements described above.

Form S-8 registration statement

In connection with this offering, we intend to file a registration statement on Form S-8 under the Securities Act covering all of the shares of our common stock subject to outstanding options and the shares of our common stock reserved for issuance under our stock plans. We expect to file this registration statement as soon as permitted under the Securities Act. However, the shares registered on Form S-8 may be subject to the volume limitations and the manner of sale, notice and public information requirements of Rule 144 and will not be eligible for resale until expiration of the lock-up and market standoff agreements to which they are subject.

Registration rights

We have granted demand, piggyback and Form S-3 registration rights to certain of our stockholders to sell our common stock. Registration of the sale of these shares under the Securities Act would result in these shares becoming freely tradable without restriction under the Securities Act immediately upon the effectiveness of the registration, except for shares purchased by affiliates. For a further description of these rights, see the section entitled “Description of capital stock—Registration rights.”

Material U.S. federal income tax consequences to non-U.S. holders

The following summary describes the material U.S. federal income tax consequences of the acquisition, ownership and disposition of our common stock acquired in this offering by Non-U.S. Holders (as defined below). This discussion does not address all aspects of U.S. federal income taxes, does not discuss the potential application of the alternative minimum tax or Medicare contribution tax on net investment income and does not deal with state or local taxes, U.S. federal gift and estate tax laws, except to the limited extent provided below, or any non-U.S. tax consequences that may be relevant to Non-U.S. Holders in light of their particular circumstances.

Special rules different from those described below may apply to certain Non-U.S. Holders that are subject to special treatment under the Code, such as:

- insurance companies, banks and other financial institutions;
- tax-exempt organizations (including private foundations) and tax-qualified retirement plans;
- foreign governments and international organizations;
- broker-dealers and traders in securities;
- U.S. expatriates and certain former citizens or long-term residents of the United States;
- persons required for U.S. federal income tax purposes to conform the timing of income accruals to their financial statements under Section 451(b) of the Code;
- persons that own, or are deemed to own, more than 5% of our capital stock;
- “controlled foreign corporations,” “passive foreign investment companies” and corporations that accumulate earnings to avoid U.S. federal income tax;
- persons that hold our common stock as part of a “straddle,” “hedge,” “conversion transaction,” “synthetic security” or integrated investment or other risk reduction strategy;
- persons who do not hold our common stock as a capital asset within the meaning of Section 1221 of the Code (generally, for investment purposes); and
- partnerships and other pass-through entities, and investors in such pass-through entities (regardless of their places of organization or formation).

Such Non-U.S. Holders are urged to consult their own tax advisors to determine the U.S. federal, state, local and other tax consequences that may be relevant to them.

Furthermore, the discussion below is based upon the provisions of the Code, and U.S. Treasury Regulations, rulings and judicial decisions thereunder as of the date hereof, and such authorities may be repealed, revoked or modified, possibly retroactively, and are subject to differing interpretations which could result in U.S. federal income tax consequences different from those discussed below. We have not requested a ruling from the Internal Revenue Service, or the IRS, with respect to the statements made and the conclusions reached in the following summary, and there can be no assurance that the IRS will agree with such statements and conclusions or will not take a contrary position regarding the tax consequences described herein, or that any such contrary position would not be sustained by a court.

PERSONS CONSIDERING THE PURCHASE OF OUR COMMON STOCK PURSUANT TO THIS OFFERING SHOULD CONSULT THEIR OWN TAX ADVISORS CONCERNING THE U.S. FEDERAL INCOME TAX CONSEQUENCES OF

ACQUIRING, OWNING AND DISPOSING OF OUR COMMON STOCK IN LIGHT OF THEIR PARTICULAR SITUATIONS AS WELL AS ANY CONSEQUENCES ARISING UNDER THE LAWS OF ANY OTHER TAXING JURISDICTION, INCLUDING ANY STATE, LOCAL OR NON-U.S. TAX CONSEQUENCES OR ANY U.S. FEDERAL NON-INCOME TAX CONSEQUENCES, AND THE POSSIBLE APPLICATION OF TAX TREATIES.

For the purposes of this discussion, a “Non-U.S. Holder” is a beneficial owner of common stock that is not a U.S. Holder or a partnership for U.S. federal income tax purposes. A “U.S. Holder” means a beneficial owner of our common stock that is, for U.S. federal income tax purposes, (a) an individual citizen or resident of the United States, (b) a corporation (or other entity taxable as a corporation for U.S. federal income tax purposes), created or organized in or under the laws of the United States, any state thereof or the District of Columbia, (c) an estate the income of which is subject to U.S. federal income taxation regardless of its source, or (d) a trust if it (1) is subject to the primary supervision of a court within the United States and one or more U.S. persons (within the meaning of Section 7701(a)(30) of the Code) have the authority to control all substantial decisions of the trust or (2) has a valid election in effect under applicable U.S. Treasury Regulations to be treated as a U.S. person.

If you are an individual non-U.S. citizen, you may, in some cases, be deemed to be a resident alien (as opposed to a nonresident alien) by virtue of being present in the United States for at least 31 days in the calendar year and for an aggregate of at least 183 days during a three-year period ending in the current calendar year. Generally, for this purpose, all the days present in the current year, one-third of the days present in the immediately preceding year, and one-sixth of the days present in the second preceding year, are counted.

Resident aliens are generally subject to U.S. federal income tax as if they were U.S. citizens. Individuals who are uncertain of their status as resident or nonresident aliens for U.S. federal income tax purposes are urged to consult their own tax advisors regarding the U.S. federal income tax consequences of the ownership or disposition of our common stock.

Distributions

We do not expect to make any distributions on our common stock in the foreseeable future. If we do make distributions on our common stock, however, such distributions made to a Non-U.S. Holder of our common stock will constitute dividends for U.S. tax purposes to the extent paid out of our current or accumulated earnings and profits (as determined under U.S. federal income tax principles). Distributions in excess of our current and accumulated earnings and profits will constitute a return of capital that is applied against and reduces, but not below zero, a Non-U.S. Holder’s adjusted tax basis in our common stock. Any remaining excess will be treated as gain realized on the sale or exchange of our common stock as described below under the section entitled “—Gain on disposition of our common stock.”

Any distribution on our common stock that is treated as a dividend paid to a Non-U.S. Holder that is not effectively connected with the holder’s conduct of a trade or business in the United States will generally be subject to withholding tax at a 30% rate or such lower rate as may be specified by an applicable income tax treaty between the United States and the Non-U.S. Holder’s country of residence. To obtain a reduced rate of withholding under a treaty, a Non-U.S. Holder generally will be required to provide the applicable withholding agent with a properly executed IRS Form W-8BEN, IRS Form W-8BEN-E or other appropriate form, certifying the Non-U.S. Holder’s entitlement to benefits under that treaty. Such form must be provided prior to the payment of dividends and must be updated periodically. If a Non-U.S. Holder holds stock through a financial institution or other agent acting on the holder’s behalf, the holder will be required to provide appropriate documentation to such agent. The holder’s agent may then be required to provide certification to the applicable withholding agent, either directly or through other intermediaries. If you are eligible for a reduced rate of U.S. withholding tax under an income tax treaty, you should consult with your own tax advisor to determine if you are able to obtain a refund of any excess amounts withheld by timely filing an appropriate claim for a refund with the IRS.

We generally are not required to withhold tax on dividends paid to a Non-U.S. Holder that are effectively connected with the holder's conduct of a trade or business within the United States (and, if required by an applicable income tax treaty, are attributable to a permanent establishment that the holder maintains in the United States) if a properly executed IRS Form W-8ECI, stating that the dividends are so connected, is furnished to us (or, if stock is held through a financial institution or other agent, to the applicable withholding agent). In general, such effectively connected dividends will be subject to U.S. federal income tax on a net income basis at the regular graduated rates applicable to U.S. persons. A corporate Non-U.S. Holder receiving effectively connected dividends may also be subject to an additional "branch profits tax," which is imposed, under certain circumstances, at a rate of 30% (or such lower rate as may be specified by an applicable treaty) on the corporate Non-U.S. Holder's effectively connected earnings and profits, subject to certain adjustments.

See also the section below entitled "—Foreign accounts" for additional withholding rules that may apply to dividends paid to certain foreign financial institutions or non-financial foreign entities.

Gain on disposition of our common stock

Subject to the discussions below under the sections entitled "—Backup withholding and information reporting" and "—Foreign accounts," a Non-U.S. Holder generally will not be subject to U.S. federal income or withholding tax with respect to gain realized on a sale or other disposition of our common stock unless (a) the gain is effectively connected with a trade or business of the holder in the United States (and, if required by an applicable income tax treaty, is attributable to a permanent establishment that the holder maintains in the United States), (b) the Non-U.S. Holder is a nonresident alien individual and is present in the United States for 183 or more days in the taxable year of the disposition and certain other conditions are met, or (c) we are or have been a "United States real property holding corporation" within the meaning of Code Section 897(c)(2) at any time within the shorter of the five-year period preceding such disposition or the holder's holding period in the common stock.

If you are a Non-U.S. Holder described in (a) above, you will be required to pay tax on the net gain derived from the sale at the regular graduated U.S. federal income tax rates applicable to U.S. persons. Corporate Non-U.S. Holders described in (a) above may also be subject to the additional branch profits tax at a 30% rate or such lower rate as may be specified by an applicable income tax treaty. If you are an individual Non-U.S. Holder described in (b) above, you will be required to pay a flat 30% tax on the gain derived from the sale, which gain may be offset by U.S. source capital losses (even though you are not considered a resident of the United States), provided you have timely filed U.S. federal income tax returns with respect to such losses. With respect to (c) above, in general, we would be a United States real property holding corporation if U.S. real property interests as defined in the Code and the U.S. Treasury Regulations comprised (by fair market value) at least half of our worldwide real property interests plus our other assets used or held for use in a trade or business. We believe that we are not, and do not anticipate becoming, a United States real property holding corporation. However, there can be no assurance that we will not become a United States real property holding corporation in the future. Even if we are treated as a U.S. real property holding corporation, gain realized by a Non-U.S. Holder on a disposition of our common stock will not be subject to U.S. federal income tax so long as (1) the Non-U.S. Holder owned, directly, indirectly or constructively, no more than five percent of our common stock at all times within the shorter of (i) the five-year period preceding the disposition or (ii) the holder's holding period and (2) our common stock is regularly traded on an established securities market, such as Nasdaq. However, there can be no assurance that our common stock will qualify as regularly traded on an established securities market.

U.S. federal estate tax

The estates of nonresident alien individuals generally are subject to U.S. federal estate tax on property with a U.S. situs. Because we are a U.S. corporation, our common stock will be U.S. situs property and, therefore, will

be included in the taxable estate of a nonresident alien decedent, unless an applicable estate tax treaty between the United States and the decedent's country of residence provides otherwise. The terms "resident" and "nonresident" are defined differently for U.S. federal estate tax purposes than for U.S. federal income tax purposes. Investors are urged to consult their own tax advisors regarding the U.S. federal estate tax consequences of the ownership or disposition of our common stock.

Backup withholding and information reporting

Generally, we or certain financial middlemen must report information to the IRS with respect to any dividends we pay on our common stock including the amount of any such dividends, the name and address of the recipient, and the amount, if any, of tax withheld. A similar report is sent to the holder to whom any such dividends are paid. Pursuant to tax treaties or certain other agreements, the IRS may make its reports available to tax authorities in the recipient's country of residence.

Dividends paid by us (or our paying agents) to a Non-U.S. Holder may also be subject to U.S. backup withholding. U.S. backup withholding generally will not apply to a Non-U.S. Holder who provides a properly executed IRS Form W-8BEN or IRS Form W-8BEN-E, as applicable, or otherwise establishes an exemption, provided that the applicable withholding agent does not have actual knowledge or reason to know the holder is a U.S. person.

Under current U.S. federal income tax law, U.S. information reporting and backup withholding requirements generally will apply to the proceeds of a disposition of our common stock effected by or through a U.S. office of any broker, U.S. or non-U.S., unless the Non-U.S. Holder provides a properly executed IRS Form W-8BEN or IRS Form W-8BEN-E, as applicable, or otherwise meets documentary evidence requirements for establishing non-U.S. person status or otherwise establishes an exemption. Generally, U.S. information reporting and backup withholding requirements will not apply to a payment of disposition proceeds to a Non-U.S. Holder where the transaction is effected outside the United States through a non-U.S. office of a non-U.S. broker. Information reporting and backup withholding requirements may, however, apply to a payment of disposition proceeds if the broker has actual knowledge, or reason to know, that the holder is, in fact, a U.S. person. For information reporting purposes, certain brokers with substantial U.S. ownership or operations will generally be treated in a manner similar to U.S. brokers.

Backup withholding is not an additional tax. If backup withholding is applied to you, you should consult with your own tax advisor to determine whether you have overpaid your U.S. federal income tax, and whether you are able to obtain a tax refund or credit of the overpaid amount.

Foreign accounts

In addition, U.S. federal withholding taxes may apply under legislation common known as the Foreign Account Tax Compliance Act, or FATCA, on certain types of payments, including dividends paid to non-U.S. financial institutions and certain other non-U.S. entities. Specifically, a 30% withholding tax may be imposed on dividends on our common stock paid to a "foreign financial institution" or a "non-financial foreign entity" (each as defined in the Code), unless (1) the foreign financial institution agrees to undertake certain diligence and reporting obligations, (2) the non-financial foreign entity either certifies it does not have any "substantial United States owners" (as defined in the Code) or furnishes identifying information regarding each substantial United States owner, or (3) the foreign financial institution or non-financial foreign entity otherwise qualifies for an exemption from these rules. The 30% federal withholding tax described in this paragraph cannot be reduced under an income tax treaty with the United States. If the payee is a foreign financial institution and is subject to the diligence and reporting requirements in (1) above, it must enter into an agreement with the U.S. Department of the Treasury requiring, among other things, that it undertake to identify accounts held by certain "specified United States persons" or "United States-owned foreign entities" (each as defined in the

Code), annually report certain information about such accounts, and withhold 30% on certain payments to non-compliant foreign financial institutions and certain other account holders. Under proposed U.S. Treasury Regulations promulgated by the Treasury Department on December 13, 2018, which state that taxpayers may rely on the proposed Treasury Regulations until final Treasury Regulations are issued, this withholding tax will not apply to the gross proceeds from any sale or disposition of our common stock. Foreign financial institutions located in jurisdictions that have an intergovernmental agreement with the United States governing FATCA may be subject to different rules.

Prospective investors should consult their tax advisors regarding the potential application of withholding under FATCA to their investment in our common stock.

EACH PROSPECTIVE INVESTOR SHOULD CONSULT ITS OWN TAX ADVISOR REGARDING THE TAX CONSEQUENCES OF PURCHASING, HOLDING AND DISPOSING OF OUR COMMON STOCK, INCLUDING THE CONSEQUENCES OF ANY PROPOSED CHANGE IN APPLICABLE LAW, AS WELL AS TAX CONSEQUENCES ARISING UNDER ANY STATE, LOCAL, NON-U.S. OR U.S. FEDERAL NON-INCOME TAX LAWS SUCH AS ESTATE AND GIFT TAX.

Underwriting

We are offering the shares of common stock described in this prospectus through a number of underwriters. J.P. Morgan Securities LLC, Cowen and Company, LLC and Credit Suisse Securities (USA) LLC are acting as joint book-running managers of the offering and as representatives of the underwriters. We have entered into an underwriting agreement with the representatives. Subject to the terms and conditions of the underwriting agreement, we have agreed to sell to the underwriters, and each underwriter has severally agreed to purchase, at the initial public offering price less the underwriting discounts and commissions set forth on the cover page of this prospectus, the number of shares of common stock listed next to its name in the following table:

Name	Number of shares
J.P. Morgan Securities LLC	
Cowen and Company, LLC	
Credit Suisse Securities (USA) LLC	
Canaccord Genuity LLC	
Total	6,700,000

The underwriters are committed to purchase all the common shares offered by us if they purchase any shares. The underwriting agreement also provides that if an underwriter defaults, the purchase commitments of non-defaulting underwriters may also be increased or the offering may be terminated.

The underwriters propose to offer the common shares directly to the public at the initial public offering price set forth on the cover page of this prospectus and to certain dealers at that price less a concession not in excess of $ per share. Any such dealers may resell shares to certain other brokers or dealers at a discount of up to $ per share from the initial public offering price. After the initial offering of the shares to the public, if all of the common shares are not sold at the initial public offering price, the underwriters may change the offering price and the other selling terms. Sales of shares made outside of the United States may be made by affiliates of the underwriters. The offering of shares by the underwriters is subject to receipt and acceptance and subject to the underwriters' right to reject any order in whole or in part.

The underwriters have an option to buy up to 1,005,000 additional shares of common stock from us to cover sales of shares by the underwriters which exceed the number of shares specified in the table above. The underwriters have 30 days from the date of this prospectus to exercise this option to purchase additional shares. If any shares are purchased with this option to purchase additional shares, the underwriters will purchase shares in approximately the same proportion as shown in the table above. If any additional shares of common stock are purchased, the underwriters will offer the additional shares on the same terms as those on which the shares are being offered.

The underwriting fee is equal to the public offering price per share of common stock less the amount paid by the underwriters to us per share of common stock. The underwriting fee is $ per share. The following table shows the per share and total underwriting discounts and commissions to be paid to the underwriters assuming both no exercise and full exercise of the underwriters' option to purchase additional shares.

	Without option to purchase additional shares exercise	With full option to purchase additional shares exercise
Per Share	$	$
Total	$	$

We estimate that the total expenses of this offering, including registration, filing and listing fees, printing fees and legal and accounting expenses, but excluding the underwriting discounts and commissions, will be approximately $2,450,000. We have agreed to reimburse the underwriters for expenses of up to $35,000 related to clearance of this offering with the Financial Industry Regulatory Authority, or FINRA.

A prospectus in electronic format may be made available on the web sites maintained by one or more underwriters, or selling group members, if any, participating in this offering. The underwriters may agree to allocate a number of shares to underwriters and selling group members for sale to their online brokerage account holders. Internet distributions will be allocated by the representatives to underwriters and selling group members that may make Internet distributions on the same basis as other allocations.

We have agreed that we will not (i) offer, pledge, announce the intention to sell, sell, contract to sell, sell any option or contract to purchase, purchase any option or contract to sell, grant any option, right or warrant to purchase or otherwise dispose of, directly or indirectly, or file with the SEC a registration statement under the Securities Act of 1933, as amended, or the Securities Act, relating to, any shares of our common stock or securities convertible into or exchangeable or exercisable for any shares of our common stock, or publicly disclose the intention to make any offer, sale, pledge, disposition or filing, or (ii) enter into any swap or other arrangement that transfers all or a portion of the economic consequences associated with the ownership of any shares of common stock or any such other securities (regardless of whether any of these transactions are to be settled by the delivery of shares of common stock or such other securities, in cash or otherwise), in each case without the prior written consent of J.P. Morgan Securities LLC for a period of 180 days after the date of this prospectus, other than the shares of our common stock to be sold hereunder and any shares of our common stock issued upon the exercise of options granted under our existing equity incentive plans.

Our directors, executive officers and shareholders have entered into lock-up agreements with the underwriters prior to the commencement of this offering pursuant to which each of these persons or entities, with limited exceptions, for a period of 180 days after the date of this prospectus, may not, without the prior written consent of J.P. Morgan Securities LLC, (1) offer, pledge, announce the intention to sell, sell, contract to sell, sell any option or contract to purchase, purchase any option or contract to sell, grant any option, right or warrant to purchase, or otherwise transfer or dispose of, directly or indirectly, any shares of our common stock or any securities convertible into or exercisable or exchangeable for our common stock (including, without limitation, common stock or such other securities which may be deemed to be beneficially owned by such directors, executive officers, managers and members in accordance with the rules and regulations of the SEC and securities which may be issued upon exercise of a stock option or warrant) or (2) enter into any swap or other agreement that transfers, in whole or in part, any of the economic consequences of ownership of the common stock or such other securities, whether any such transaction described in clause (1) or (2) above is to be settled by delivery of common stock or such other securities, in cash or otherwise, or (3) make any demand for or exercise any right with respect to the registration of any shares of our common stock or any security convertible into or exercisable or exchangeable for our common stock.

The restrictions described in the immediately preceding paragraph do not apply to, among other items, transfers or dispositions of shares of common stock:

- as a bona fide gift, including bona fide gifts to a charity or educational institution, or for bona fide estate planning purposes;
- to any trust for the direct or indirect benefit of the party subject to the lock-up restrictions or the immediate family of such person;

- to any corporation, partnership, limited liability company or other entity under the ownership of the party subject to the lock-up restrictions or the immediate family of such person;
- by will, other testamentary document or intestate succession to the legal representative, heir, beneficiary or a member of the immediate family of the party subject to the lockup restrictions;
- as distributions to partners, members or stockholders of the party subject to the lock-up restrictions; and
- as transfers to affiliates, investment funds or other entities controlled or managed by the party subject to the lock-up restrictions; and

provided that in the case of any transfer or distribution pursuant to the six subclauses above, (i) each transferee, donee or distributee shall sign and deliver a lock-up letter in the form executed by the party subject to the lock up restrictions and (ii) no filing or other public announcement under Section 16(a)of the Exchange Act of 1934, as amended, or the Exchange Act, shall be required or shall be voluntarily made during the restricted period (other than a filing on Form 5 or a required filing on a Schedule 13F or 13G);

- the transfer pursuant to a bona fide third-party tender offer, merger, consolidation or other similar transaction made to all holders of our common stock involving a change of control; provided that in the event such tender offer, merger, consolidation or other such transaction is not completed, the shares of our common stock shall remain subject to the lock-up restrictions;
- the exercise of outstanding warrants or options to purchase shares of common stock granted under any stock incentive plan or stock purchase plan of ours, provided that the underlying shares shall continue to be subject to the lock-up restrictions;
- the establishment of a trading plan pursuant to Rule 10b5-1 under the Exchange Act for the transfer of shares of common stock, provided that (i) such plan does not provide for the transfer of shares of common stock during the restricted period and (ii) no filing under the Exchange Act or other public announcement shall be required or voluntarily made by or on behalf of the party subject to the lock-up restrictions regarding the establishment of such plan;
- the transfer or disposition of shares of common stock acquired in this offering or on the open market following this offering, provided that no filing under the Exchange Act or other public announcement shall be required or voluntarily made in connection with such transfer or disposition during the restricted period (other than a required filing on a Schedule 13F or 13G);
- transfers or surrenders to us of shares of common stock pursuant to any contractual arrangement that provides us with an option to repurchase such shares in connection with the termination of the party subject to the lock-up's employment or service relationship with us, or pursuant to a right of first refusal with respect to transfers of such shares of common stock or other securities, or on a cashless or "net exercise" basis or to cover tax withholding obligations of the party subject to the lock-up, in connection with the vesting or exercise of such shares of common stock or other securities, provided that any filing under Section 16 of the Exchange Act shall clearly indicate in the footnotes thereto that the filing relates to such circumstances described above and no other public announcement shall be required or voluntarily made in connection with such transfers or surrenders; and
- transfers or dispositions of shares of common stock by operation of law pursuant to a qualified domestic order or in connection with a divorce settlement or other court order, provided that the recipient of such shares shall execute and deliver to J.P. Morgan Securities LLC a lock-up letter in the form executed by the party subject to the lock-up restrictions, provided, further that any filing under Section 16 of the Exchange Act shall clearly indicate in the footnotes that the filing relates to the circumstances described above and no other public announcement shall be required or voluntarily made in connection with such transfer or disposition.

We have agreed to indemnify the several underwriters against certain liabilities, including liabilities under the Securities Act.

We have applied to list our common stock on Nasdaq under the symbol “STOK.”

In connection with this offering, the underwriters may engage in stabilizing transactions, which involves making bids for, purchasing and selling shares of common stock in the open market for the purpose of preventing or retarding a decline in the market price of the common stock while this offering is in progress. These stabilizing transactions may include making short sales of the common stock, which involves the sale by the underwriters of a greater number of shares of common stock than they are required to purchase in this offering, and purchasing shares of common stock on the open market to cover positions created by short sales. Short sales may be “covered” shorts, which are short positions in an amount not greater than the underwriters’ option to purchase additional shares referred to above, or may be “naked” shorts, which are short positions in excess of that amount. The underwriters may close out any covered short position either by exercising their option to purchase additional shares, in whole or in part, or by purchasing shares in the open market. In making this determination, the underwriters will consider, among other things, the price of shares available for purchase in the open market compared to the price at which the underwriters may purchase shares through the option to purchase additional shares. A naked short position is more likely to be created if the underwriters are concerned that there may be downward pressure on the price of the common stock in the open market that could adversely affect investors who purchase in this offering. To the extent that the underwriters create a naked short position, they will purchase shares in the open market to cover the position.

The underwriters have advised us that, pursuant to Regulation M of the Securities Act, they may also engage in other activities that stabilize, maintain or otherwise affect the price of the common stock, including the imposition of penalty bids. This means that if the representatives of the underwriters purchase common stock in the open market in stabilizing transactions or to cover short sales, the representatives can require the underwriters that sold those shares as part of this offering to repay the underwriting discount received by them.

These activities may have the effect of raising or maintaining the market price of the common stock or preventing or retarding a decline in the market price of the common stock, and, as a result, the price of the common stock may be higher than the price that otherwise might exist in the open market. If the underwriters commence these activities, they may discontinue them at any time. The underwriters may carry out these transactions on the Nasdaq in the over-the-counter market or otherwise.

Prior to this offering, there has been no public market for our common stock. The initial public offering price will be determined by negotiations between us and the representatives of the underwriters. In determining the initial public offering price, we and the representatives of the underwriters expect to consider a number of factors including:

- the information set forth in this prospectus and otherwise available to the underwriters;
- our prospects and the history and prospects for the industry in which we compete;
- an assessment of our management;
- our prospects for future earnings;
- the general condition of the securities markets at the time of this offering;
- the recent market prices of, and demand for, publicly traded common stock of generally comparable companies; and
- other factors deemed relevant by the underwriters and us.

Neither we nor the underwriters can assure investors that an active trading market will develop for our common shares, or that the shares will trade in the public market at or above the initial public offering price.

Other relationships

The underwriters and their respective affiliates are full service financial institutions engaged in various activities, which may include sales and trading, commercial and investment banking, advisory, investment management, investment research, principal investment, hedging, market making, brokerage and other financial and non-financial activities and services. Certain of the underwriters and their respective affiliates may in the future provide various commercial banking, financial advisory or investment banking advice or other services in the ordinary course of their business, for which they will receive customary fees and commissions. In addition, from time to time, certain of the underwriters and their respective affiliates, officers, directors and employees may effect transactions for their own account or the account of customers, and hold on behalf of themselves or their customers, long or short positions in our debt or equity securities or loans, and may do so in the future. The underwriters and their respective affiliates may also communicate independent investment recommendations, market color or trading ideas and/or publish or express independent research views in respect of our securities and may at any time hold, or recommend to clients that they should acquire, long and/or short positions in our securities.

Selling restrictions

General

Other than in the United States, no action has been taken by us or the underwriters that would permit a public offering of the securities offered by this prospectus in any jurisdiction where action for that purpose is required. The securities offered by this prospectus may not be offered or sold, directly or indirectly, nor may this prospectus or any other offering material or advertisements in connection with the offer and sale of any such securities be distributed or published in any jurisdiction, except under circumstances that will result in compliance with the applicable rules and regulations of that jurisdiction. Persons into whose possession this prospectus comes are advised to inform themselves about and to observe any restrictions relating to the offering and the distribution of this prospectus. This prospectus does not constitute an offer to sell or a solicitation of an offer to buy any securities offered by this prospectus in any jurisdiction in which such an offer or a solicitation is unlawful.

Canada

The shares may be sold only to purchasers purchasing, or deemed to be purchasing, as principal that are accredited investors, as defined in National Instrument 45-106 *Prospectus Exemptions* or subsection 73.3(1) of the *Securities Act* (Ontario), and are permitted clients, as defined in National Instrument 31-103 *Registration Requirements, Exemptions and Ongoing Registrant Obligations*. Any resale of the shares must be made in accordance with an exemption from, or in a transaction not subject to, the prospectus requirements of applicable securities laws.

Securities legislation in certain provinces or territories of Canada may provide a purchaser with remedies for rescission or damages if this prospectus (including any amendment thereto) contains a misrepresentation, provided that the remedies for rescission or damages are exercised by the purchaser within the time limit prescribed by the securities legislation of the purchaser's province or territory. The purchaser should refer to any applicable provisions of the securities legislation of the purchaser's province or territory for particulars of these rights or consult with a legal advisor.

Pursuant to section 3A.3 of National Instrument 33-105 *Underwriting Conflicts* (NI 33-105), the representatives are not required to comply with the disclosure requirements of NI 33-105 regarding underwriter conflicts of interest in connection with this offering.

European Economic Area

In relation to each Member State of the European Economic Area (each, a "Relevant Member State"), no offer of shares may be made to the public in that Relevant Member State other than:

A. to any legal entity which is a qualified investor as defined in the Prospectus Directive;

B. to fewer than 100 or, if the Relevant Member State has implemented the relevant provision of the 2010 PD Amending Directive, 150, natural or legal persons (other than qualified investors as defined in the Prospectus Directive), as permitted under the Prospectus Directive, subject to obtaining the prior consent of the representatives; or

C. in any other circumstances falling within Article 3(2) of the Prospectus Directive,

provided that no such offer of shares shall require the Company or the representatives to publish a prospectus pursuant to Article 3 of the Prospectus Directive or supplement a prospectus pursuant to Article 16 of the Prospectus Directive.

Each person in a Relevant Member State who initially acquires any shares or to whom any offer is made will be deemed to have represented, acknowledged and agreed that it is a "qualified investor" within the meaning of the law in that Relevant Member State implementing Article 2(1)(e) of the Prospectus Directive. In the case of any shares being offered to a financial intermediary as that term is used in Article 3(2) of the Prospectus Directive, each such financial intermediary will be deemed to have represented, acknowledged and agreed that the shares acquired by it in the offer have not been acquired on a non-discretionary basis on behalf of, nor have they been acquired with a view to their offer or resale to, persons in circumstances which may give rise to an offer of any shares to the public other than their offer or resale in a Relevant Member State to qualified investors as so defined or in circumstances in which the prior consent of the representatives has been obtained to each such proposed offer or resale.

The Company, the representatives and their affiliates will rely upon the truth and accuracy of the foregoing representations, acknowledgements and agreements.

This prospectus has been prepared on the basis that any offer of shares in any Relevant Member State will be made pursuant to an exemption under the Prospectus Directive from the requirement to publish a prospectus for offers of shares. Accordingly, any person making or intending to make an offer in that Relevant Member State of shares which are the subject of the offering contemplated in this prospectus may only do so in circumstances in which no obligation arises for the Company or any of the underwriters to publish a prospectus pursuant to Article 3 of the Prospectus Directive in relation to such offer. Neither the Company nor the underwriters have authorized, nor do they authorize, the making of any offer of shares in circumstances in which an obligation arises for the Company or the underwriters to publish a prospectus for such offer.

For the purpose of the above provisions, the expression "an offer to the public" in relation to any shares in any Relevant Member State means the communication in any form and by any means of sufficient information on the terms of the offer and the shares to be offered so as to enable an investor to decide to purchase or subscribe the shares, as the same may be varied in the Relevant Member State by any measure implementing the Prospectus Directive in the Relevant Member State and the expression "Prospectus Directive" means Directive 2003/71/EC (including the 2010 PD Amending Directive, to the extent implemented in the Relevant

Member States) and includes any relevant implementing measure in the Relevant Member State and the expression "2010 PD Amending Directive" means Directive 2010/73/EU.

Hong Kong

The shares may not be offered or sold by means of any document other than (i) in circumstances that do not constitute an offer to the public within the meaning of the Companies Ordinance (Cap. 32, Laws of Hong Kong), or (ii) to "professional investors" within the meaning of the Securities and Futures Ordinance (Cap. 571, Laws of Hong Kong) and any rules made thereunder, or (iii) in other circumstances that do not result in the document being a "prospectus" within the meaning of the Companies Ordinance (Cap. 32, Laws of Hong Kong), and no advertisement, invitation or document relating to the shares may be issued or may be in the possession of any person for the purpose of issue (in each case whether in Hong Kong or elsewhere), which is directed at, or the contents of which are likely to be accessed or read by, the public in Hong Kong (except if permitted to do so under the laws of Hong Kong) other than with respect to shares that are or are intended to be disposed of only to persons outside Hong Kong or only to "professional investors" within the meaning of the Securities and Futures Ordinance (Cap. 571, Laws of Hong Kong) and any rules made thereunder.

Japan

The securities have not been and will not be registered under the Financial Instruments and Exchange Law of Japan, or the Financial Instruments and Exchange Law, and each underwriter has agreed that it will not offer or sell any securities, directly or indirectly, in Japan or to, or for the benefit of, any resident of Japan (which term, as used in this prospectus means any person resident in Japan, including any corporation or other entity organized under the laws of Japan) or to others for re-offering or resale, directly or indirectly, in Japan or to a resident of Japan, except pursuant to an exemption from the registration requirements of, and otherwise in compliance with, the Financial Instruments and Exchange Law and any other applicable laws, regulations and ministerial guidelines of Japan.

Singapore

This prospectus has not been registered as a prospectus with the Monetary Authority of Singapore. Accordingly, this prospectus and any other document or material in connection with the offer or sale, or invitation for subscription or purchase, of the shares may not be circulated or distributed, nor may the shares be offered or sold, or be made the subject of an invitation for subscription or purchase, whether directly or indirectly, to persons in Singapore other than (i) to an institutional investor under Section 274 of the Securities and Futures Act, Chapter 289 of Singapore, or the SFA, (ii) to a relevant person, or any person pursuant to Section 275(1A), and in accordance with the conditions specified in Section 275 of the SFA or (iii) otherwise pursuant to, and in accordance with the conditions of, any other applicable provision of the SFA.

Where the shares are subscribed or purchased under Section 275 by a relevant person that is: (a) a corporation (which is not an accredited investor) the sole business of which is to hold investments and the entire share capital of which is owned by one or more individuals, each of whom is an accredited investor; or (b) a trust (where the trustee is not an accredited investor) whose sole purpose is to hold investments and each beneficiary is an accredited investor, shares, debentures and units of shares and debentures of that corporation or the beneficiaries' rights and interest in that trust shall not be transferable for six months after that corporation or that trust has acquired the shares under Section 275 except: (1) to an institutional investor under Section 274 of the SFA or to a relevant person, or any person pursuant to Section 275(1A), and in accordance with the conditions specified in Section 275 of the SFA; (2) where no consideration is given for the transfer; or (3) by operation of law.

Switzerland

The shares may not be publicly offered in Switzerland and will not be listed on the SIX Swiss Exchange, or SIX, or on any other stock exchange or regulated trading facility in Switzerland. This document has been prepared without regard to the disclosure standards for issuance prospectuses under art. 652a or art. 1156 of the Swiss Code of Obligations or the disclosure standards for listing prospectuses under art. 27 ff. of the SIX Listing Rules or the listing rules of any other stock exchange or regulated trading facility in Switzerland. Neither this document, nor any other offering or marketing material relating to the shares or this offering, may be publicly distributed or otherwise made publicly available in Switzerland. Neither this document nor any other offering or marketing material relating to this offering, the Company, or the shares has been or will be filed with or approved by any Swiss regulatory authority. In particular, this document will not be filed with, and the offer of shares will not be supervised by, the Swiss Financial Market Supervisory Authority FINMA, or FINMA, and the offer of shares has not been and will not be authorized under the Swiss Federal Act on Collective Investment Schemes, or CISA. The investor protection afforded to acquirers of interests in collective investment schemes under the CISA does not extend to acquirers of shares.

United Arab Emirates

This prospectus relates to an Exempt Offer in accordance with the Offered Securities Rules of the Dubai Financial Services Authority, or DFSA. This prospectus is intended for distribution only to persons of a type specified in the Offered Securities Rules of the DFSA. It must not be delivered to, or relied on by, any other person. The DFSA has no responsibility for reviewing or verifying any documents in connection with Exempt Offers. The DFSA has not approved this prospectus nor taken steps to verify the information set forth herein and has no responsibility for this prospectus. The shares to which this prospectus relates may be illiquid and/or subject to restrictions on their resale. Prospective purchasers of the shares offered should conduct their own due diligence on the shares. If you do not understand the contents of this prospectus, you should consult an authorized financial advisor.

United Kingdom

This document is being distributed only to, and is directed only at, and any offer subsequently made may only be directed at persons who are “qualified investors” (as defined in the Prospectus Directive) (i) who have professional experience in matters relating to investments falling within Article 19 (5) of the Financial Services and Markets Act 2000 (Financial Promotion) Order 2005, as amended (the “Order”) and/or (ii) who are high net worth companies (or persons to whom it may otherwise be lawfully communicated) falling within Article 49 (2) (a) to (d) of the Order (all such persons together being referred to as “relevant persons”).

Any person in the United Kingdom that is not a relevant person should not act or rely on the information included in this document or use it as basis for taking any action. In the United Kingdom, any investment or investment activity that this document relates to may be made or taken exclusively by relevant persons. Any person in the United Kingdom that is not a relevant person should not act or rely on this document or any of its contents.

Legal matters

The validity of the shares of common stock offered by this prospectus will be passed upon for us by Fenwick & West LLP, San Francisco, California. Certain legal matters relating to the offering will be passed upon for the underwriters by Davis Polk & Wardwell LLP, New York, New York. Fenwick & West LLP beneficially owns an aggregate of 2,525 shares of our common stock.

Experts

The consolidated financial statements of Stoke Therapeutics, Inc. as of December 31, 2018 and 2017, and for each of the years in the two-year period ended December 31, 2018, have been included herein and in the registration statement in reliance upon the report of KPMG LLP, an independent registered public accounting firm, appearing elsewhere herein, and upon the authority of said firm as experts in accounting and auditing.

Where you can find additional information

We have filed with the SEC a registration statement on Form S-1 under the Securities Act of 1933, as amended, with respect to the shares of common stock offered hereby. This prospectus, which constitutes a part of the registration statement, does not contain all of the information set forth in the registration statement or the exhibits filed therewith. For further information about us and the common stock offered hereby, reference is made to the registration statement and the exhibits filed therewith. Statements contained in this prospectus concerning the contents of any contract or any other document are not necessarily complete. If a contract or document has been filed as an exhibit to the registration statement, please see the copy of the contract or document that has been filed for the complete contents of that contract or document. Each statement in this prospectus relating to a contract or document filed as an exhibit is qualified in all respects by the filed exhibit. The exhibits to the registration statement should be reviewed for the complete contents of these contracts and documents.

We currently do not file periodic reports with the SEC. Upon the completion of this offering, we will be required to file periodic reports, proxy statements and other information with the SEC pursuant to the Securities Exchange Act of 1934, as amended. The SEC maintains a website that contains reports, proxy and information statements and other information regarding registrants that file electronically with the SEC. The address of the website is www.sec.gov.

We also maintain a website at www.stoketherapeutics.com. Upon completion of this offering, you may access these materials at our website free of charge as soon as reasonably practicable after they are electronically filed with, or furnished to, the SEC. The information contained in, or that can be accessed through, our website is not part of, and is not incorporated into, this prospectus.

Index to financial statements

Report of independent registered public accounting firm

To the Stockholders and Board of Directors
Stoke Therapeutics, Inc.:

Opinion on the Consolidated Financial Statements

We have audited the accompanying consolidated balance sheets of Stoke Therapeutics, Inc., and subsidiary (together, the Company) as of December 31, 2018 and 2017, the related consolidated statements of operations, stockholders' equity (deficit), and cash flows for each of the years in the two-year period ended December 31, 2018, and the related notes (collectively, the consolidated financial statements). In our opinion, the consolidated financial statements present fairly, in all material respects, the financial position of the Company as of December 31, 2018 and 2017, and the results of its operations and its cash flows for each of the years in the two-year period ended December 31, 2018, in conformity with U.S. generally accepted accounting principles.

Basis for Opinion

These consolidated financial statements are the responsibility of the Company's management. Our responsibility is to express an opinion on these consolidated financial statements based on our audits. We are a public accounting firm registered with the Public Company Accounting Oversight Board (United States) (PCAOB) and are required to be independent with respect to the Company in accordance with the U.S. federal securities laws and the applicable rules and regulations of the Securities and Exchange Commission and the PCAOB.

We conducted our audits in accordance with the standards of the PCAOB. Those standards require that we plan and perform the audit to obtain reasonable assurance about whether the consolidated financial statements are free of material misstatement, whether due to error or fraud. Our audits included performing procedures to assess the risks of material misstatement of the consolidated financial statements, whether due to error or fraud, and performing procedures that respond to those risks. Such procedures included examining, on a test basis, evidence regarding the amounts and disclosures in the consolidated financial statements. Our audits also included evaluating the accounting principles used and significant estimates made by management, as well as evaluating the overall presentation of the consolidated financial statements. We believe that our audits provide a reasonable basis for our opinion.

/s/ KPMG LLP

We have served as the Company's auditor since 2019.

Boston, MA
March 26, 2019, except as to notes 1 and 14, as to which the date is June 7, 2019

Stoke Therapeutics, Inc.
Consolidated balance sheets
(in thousands, except share and per share amounts)

	As of December 31,	
	2018	**2017**
Assets		
Current assets:		
Cash and cash equivalents	$105,399	$ 1,797
Prepaid expenses and other current assets	548	113
Interest receivable	196	—
Total current assets	106,143	1,910
Restricted cash	204	56
Property and equipment, net	1,192	473
Total assets	$107,539	$ 2,439
Liabilities and stockholders' equity (deficit)		
Current liabilities:		
Accounts payable	$ 1,071	$ 141
Accrued and other current liabilities	1,396	574
Simple agreement for future equity	—	3,000
Total current liabilities	2,467	3,715
Long term liabilities	4	16
Total liabilities	2,471	3,731
Commitments and contingencies (Note 7)		
Stockholders' equity (deficit)		
Convertible Preferred Stock, par value of $0.0001 per share; 225,584,874 and 59,575,576 shares authorized, 22,677,585 and 4,980,168 shares issued and outstanding as of December 31, 2018 and 2017, respectively; aggregate liquidation preference of $130,850 at December 31, 2018	2	—
Common stock, par value of $0.0001 per share; 278,527,249 and 79,000,000 shares authorized, 727,413 and 670,090 shares issued and outstanding as of December 31, 2018 and 2017, respectively	—	—
Additional paid-in capital	130,776	11,897
Accumulated deficit	(25,710)	(13,189)
Total stockholders' equity (deficit)	105,068	(1,292)
Total liabilities and stockholders' equity (deficit)	$107,539	$ 2,439

The accompanying notes are an integral part of these consolidated financial statements.

Stoke Therapeutics, Inc.
Consolidated statements of operations
(in thousands, except share and per share amounts)

	Year Ended December 31,	
	2018	**2017**
Revenue	$ —	$ —
Operating expenses:		
Research and development	8,371	3,598
General and administrative	4,410	1,956
Total operating expenses	12,781	5,554
Loss from operations	(12,781)	(5,554)
Other income (expense):		
Interest income	270	—
Other expense, net	(10)	(4)
Total other income (expense)	260	(4)
Net loss	(12,521)	$ (5,558)
Net loss per share attributable to common stockholders—basic and diluted	$ (17.65)	$ (8.29)
Weighted average common shares outstanding—basic and diluted	709,336	670,090
Pro forma net loss per share, basic and diluted (Notes 2 and 11)	$ (0.98)	
Weighted-average shares used in computing pro forma net loss per share, basic and diluted (Notes 2 and 11)	12,784,811	

The accompanying notes are an integral part of these consolidated financial statements.

Stoke Therapeutics, Inc.
Consolidated statements of stockholders' equity (deficit)
(in thousands, except share and per share amounts)

	Convertible Preferred Stock		Common Stock				
	Shares	Amount	Shares	Amount	Additional paid-in capital	Accumulated deficit	Stockholders' equity (Deficit)
Balance as of December 31, 2016	3,719,366	$ —	670,090	$ —	$ 8,896	$ (7,631)	$ 1,265
Stock-based compensation	—	—	—	—	26	—	26
Issuance of convertible Preferred Stock, net of issuance costs of $25	1,260,802	—	—	—	2,975	—	2,975
Net loss	—	—	—	—	—	(5,558)	(5,558)
Balance as of December 31, 2017	4,980,168	—	670,090	—	11,897	(13,189)	(1,292)
Stock-based compensation	—	—	—	—	240	—	240
Issuance of common stock	—	—	57,323	—	19	—	19
Issuance of convertible Preferred Stock, net of issuance costs of $378	17,697,417	2	—	—	118,620	—	118,622
Net loss	—	—	—	—	—	(12,521)	(12,521)
Balance as of December 31, 2018	22,677,585	$ 2	727,413	$ —	$ 130,776	$ (25,710)	$ 105,068

The accompanying notes are an integral part of these consolidated financial statements.

Stoke Therapeutics, Inc.
Consolidated statements of cash flows
(in thousands)

	Year Ended December 31,	
	2018	**2017**
Cash flows from operating activities:	$ (12,521)	$(5,558)
Net loss		
Adjustments to reconcile net loss to net cash used in operating activities:		
Depreciation	214	113
Stock-based compensation	240	26
Loss on disposal of property and equipment	10	4
Changes in assets and liabilities:		
Prepaid expenses and other current assets	(632)	(72)
Accounts payable and accrued liabilities	1,735	100
Deferred rent	(10)	3
Net cash used in operating activities	(10,964)	(5,384)
Cash flows from investing activities:		
Purchases of property and equipment	(935)	(113)
Proceeds from sale of property and equipment	10	—
Net cash used in investing activities	(925)	(113)
Cash flows from financing activities:		
Proceeds from simple agreement for future equity	—	3,000
Proceeds from issuance of convertible Preferred Stock	116,000	3,000
Preferred Stock issuance costs	(378)	(25)
Proceeds from the issuance of common stock	19	—
Other	(2)	(1)
Net cash provided by financing activities	115,639	5,974
Net increase in cash, cash equivalents and restricted cash	103,750	477
Cash, cash equivalents and restricted cash—beginning of year	1,853	1,376
Cash, cash equivalents and restricted cash—end of year	$105,603	$ 1,853
Supplemental disclosure of non-cash investing and financing activities:		
Property and equipment included in accrued expenses and accounts payable	$ 19	$ —
Issuance of convertible Preferred Stock in exchange for simple agreement for future equity	$ 3,000	$ —

The accompanying notes are an integral part of these consolidated financial statements.

Stoke Therapeutics, Inc. and subsidiaries
Notes to consolidated financial statements
(in thousands, except share and per share amounts)

1. Nature of the business and basis of presentation

Organization

Stoke Therapeutics, Inc. (the Company) was founded in June 2014 and was incorporated under the laws of the State of Delaware. The Company is an early-stage biopharmaceutical company pioneering a new way to treat the underlying causes of severe genetic diseases by precisely upregulating protein expression.

On June 6, 2019, the Company effected a 9.95-for-one reverse split of the Company's issued and outstanding common and convertible preferred stock. Upon the effectiveness of the reverse stock split, (i) all shares of outstanding common stock and convertible preferred stock were adjusted; (ii) the number of shares of common stock for which each outstanding option to purchase common stock is exercisable were adjusted; and (iii) the exercise price of each outstanding option to purchase common stock were adjusted. All of the outstanding common stock share numbers (including shares of common stock subject to the Company's options and as converted for the outstanding convertible preferred stock shares), share prices, exercise prices and per share amounts contained in the consolidated financial statements have been retroactively adjusted in the consolidated financial statements to reflect this reverse stock split for all periods presented. The par value per share and the authorized number of shares of common stock and convertible preferred stock were not adjusted as a result of the reverse stock split.

Uncertainties

The Company is subject to risks and uncertainties common to early-stage companies in the biotechnology industry including, but not limited to, development by competitors of new technological innovations, dependence on key personnel, protection of proprietary technology, compliance with government regulations and ability to secure additional capital to fund operations. Product candidates currently under development will require significant additional research and development efforts, including extensive preclinical and clinical testing and regulatory approval prior to commercialization. These efforts require significant amounts of additional capital, adequate personnel and infrastructure and extensive compliance-reporting capabilities. Even if the Company's product development efforts are successful, it is uncertain when, if ever, the Company will realize significant revenue from product sales.

Liquidity

The Company expects that its operating losses and negative cash flows will continue for the foreseeable future. As of the issuance date of its consolidated financial statements as of and for the year ended December 31, 2018, the Company expects that its cash and cash equivalents will be sufficient to fund its operating expenses and capital expenditure requirements through at least twelve months from the issuance date of its consolidated financial statements. The future viability of the Company beyond that date is dependent on its ability to raise additional capital to finance its operations.

The Company plans to seek additional funding through public or private equity offerings, debt financings, other collaborations, strategic alliances and licensing arrangements. The Company may not be able to obtain financing on acceptable terms, or at all, and the Company may not be able to enter into strategic alliances or other arrangements on favorable terms, or at all. The terms of any financing may adversely affect the holdings or the rights of the Company's stockholders. If the Company is unable to obtain funding, the Company could be required to delay, reduce or eliminate research and development programs, product portfolio expansion or future commercialization efforts, which could adversely affect its business prospects.

2. Summary of significant accounting policies and recent accounting pronouncements

Basis of presentation and consolidation

The accompanying consolidated financial statements have been prepared in accordance with generally accepted accounting principles (GAAP), and include the accounts of Stoke Therapeutics, Inc. and its wholly-owned subsidiary. All intercompany transactions between and among its consolidated subsidiary have been eliminated.

Unaudited Pro Forma Information

In the accompanying statements of operations, the unaudited pro forma basic and diluted net loss per share attributable to common stockholders for the year ended December 31, 2018 has been prepared to give effect to the automatic conversion of all outstanding shares of convertible preferred stock as if the Company's proposed initial public offering had occurred on the later of January 1, 2018 or the issuance date of the convertible preferred stock.

Use of estimates

The preparation of consolidated financial statements in conformity with GAAP requires management to make estimates and assumptions that affect the reported amounts of assets, liabilities, equity, expenses and disclosure of contingent assets and liabilities. Estimates are periodically reviewed in light of changes in circumstances, facts and experience. Actual results could differ from our estimates.

Cash and cash equivalents

The Company considers all highly liquid investments with an original maturity of three months or less at the date of purchase to be cash equivalents. The Company deposits its cash in checking, sweep account and money market accounts.

Restricted cash

At December 31, 2018, restricted cash consisted of money market accounts collateralizing a letter of credit issued as a security deposit in connection with the Company's lease of its corporate facilities.

The following table reconciles cash and cash equivalents and restricted cash per the consolidated balance sheet to the statement of cash flows:

	As of December 31,	
	2018	**2017**
Cash and cash equivalents	$ 105,399	1,797
Restricted cash	204	56
	$ 105,603	1,853

Concentration of credit risk

Financial instruments that potentially expose the Company to concentrations of credit risk primarily consist of cash and cash equivalents. The Company maintains its cash and cash equivalents at an accredited financial institution in amounts that exceed federally insured limits. The Company does not believe that it is subject to unusual credit risk beyond the normal credit risk associated with commercial banking relationships.

Fair value of financial instruments

Accounting Standards Codification (ASC) Topic 820, *Fair Value Measurement* (ASC 820), establishes a fair value hierarchy for instruments measured at fair value that distinguishes between assumptions based on market data

(observable inputs) and the Company's own assumptions (unobservable inputs). Observable inputs are those that market participants would use in pricing the asset or liability based on market data obtained from sources independent of the Company. Unobservable inputs are inputs that reflect the Company's assumptions about the inputs that market participants would use in pricing the asset or liability and are developed based on the best information available in the circumstances. ASC 820 identifies fair value as the exchange price, or exit price, representing the amount that would be received to sell an asset or paid to transfer a liability in an orderly transaction between market participants. As a basis for considering market participant assumptions in fair value measurements, ASC 820 establishes a three-tier value hierarchy that distinguishes between the following:

Level 1—Quoted market prices in active markets for identical assets or liabilities.

Level 2—Inputs other than Level 1 inputs that are either directly or indirectly observable, such as quoted market prices, interest rates and yield curves.

Level 3—Unobservable inputs developed using estimates of assumptions developed by the Company, which reflect those that a market participant would use.

To the extent the valuation is based on models or inputs that are less observable or unobservable in the market, the determination of fair values requires more judgment. Accordingly, the degree of judgment exercised by the Company in determining fair value is greatest for instruments categorized as Level 3. A financial instrument's level within the fair value hierarchy is based on the lowest level of any input that is significant to the fair value measurement.

Deferred offering costs

The Company capitalizes certain legal, professional accounting and other third-party fees that are directly associated with in-process equity financings as deferred offering costs until such financings are consummated. After consummation of the equity financing, these costs are recorded in stockholders' equity (deficit) as a reduction of additional paid-in capital generated as a result of the offering. Should the in-process equity financing be abandoned the deferred offering costs will be expensed immediately as a charge to operating expenses in the consolidated statements of operations.

Property and equipment

Property and equipment are initially recorded at cost less accumulated depreciation. Cost includes the acquisition costs and all costs necessary to bring the asset to the location and working condition necessary for its intended use. Depreciation expense is recognized using the straight-line method over the estimated useful life of each asset. Upon retirement or sale, the cost of assets disposed of and the related accumulated depreciation are removed from the accounts and any resulting gain or loss is included in the accompanying consolidated statements of operations. Expenditures for normal, recurring or periodic repairs and maintenance related to property and equipment are charged to expense as incurred. The cost for planned major maintenance activities, including the related acquisition or construction of assets, is capitalized if it will result in future economic benefits.

Estimated useful lives for property and equipment are as follows:

Property and equipment	Estimated useful life
Computer and office equipment	3-5 years
Laboratory equipment and Furniture and fixtures	5-7 years
Leasehold improvements	Lesser of estimated useful life or remaining lease term

Impairment of long-lived assets

The Company reviews the recoverability of its long-lived assets when events or changes in circumstances occur that indicate that the carrying value of the assets may not be recoverable. The assessment of possible impairment is based on the ability to recover the carrying value of the assets from the expected future cash flows (undiscounted and without interest expense) of the related operations. If these cash flows are less than the carrying value of such assets, an impairment loss for the difference between the estimated fair value and carrying value is recorded. There were no impairment losses recognized during the years ended December 31, 2018 and 2017.

Research and development costs

Research and development costs are expensed as incurred. Research and development expenses are comprised of costs incurred in performing research and development activities, including salaries and benefits, facilities costs, depreciation, third-party license fees, and costs related to third parties engaged to conduct preclinical research development activities.

The Company has entered into various research and development contracts with research institutions and other companies to conduct research on its behalf. These agreements are generally cancellable. The Company records accruals for estimated ongoing research costs. When evaluating the adequacy of the accrued liabilities, the Company analyzes progress of the studies, including the phase or completion of events, invoices received and contracted costs. Significant judgments and estimates may be made in determining the accrued balances at the end of any reporting period. Actual results could differ from the Company's estimates. The Company's historical accrual estimates have not been materially different from the actual costs.

Stock options

The Company measures its stock-based awards granted based on the estimated fair values of the awards and recognizes the compensation for employees and nonemployees over the requisite service period. The Company uses the Black-Scholes option-pricing model to estimate the fair value of its stock-based awards. The Company has elected the practical expedient to use the midpoint between vesting date and the contractual term as the expected term for certain awards with service or performance conditions. Stock-based compensation is recognized using the straight-line method. Forfeitures of unvested stock-based awards are accounted for when they occur.

Patent costs

All patent-related costs incurred in connection with filing and prosecuting patent applications are expensed as incurred due to the uncertainty about the recovery of the expenditure. Amounts incurred are classified as general and administrative expenses in the accompanying consolidated statements of operations.

Rent expense

The Company's real estate operating lease provides for scheduled annual rent increases throughout the lease term. The Company recognizes the effects of the scheduled rent increases on a straight-line basis over the full term of the lease. Tenant improvement allowances, if any, provided by a landlord are recorded as deferred rent and amortized as reduction to rent expense over the lease term.

Income taxes

The Company accounts for income taxes using the asset and liability method, which requires the recognition of deferred tax assets and liabilities for the estimated future tax consequences attributable to temporary differences between the consolidated financial statement carrying amounts of existing assets and liabilities and their respective tax base. Deferred tax assets and liabilities are determined on the basis of the differences

between the consolidated financial statements and tax basis of assets and liabilities using enacted tax rates in effect for the year in which the temporary differences are expected to be settled or recovered. Changes in deferred tax assets and liabilities are recorded in the provision for income taxes. The Company assesses the likelihood that its deferred tax assets will be recovered from future taxable income and, to the extent it believes, based upon the weight of available evidence that it is more likely than not that all or a portion of the deferred tax assets will not be realized, a valuation allowance is established through a charge to income tax expense. Potential for recovery of deferred tax assets is evaluated by estimating the future taxable profits expected and considering prudent and feasible tax planning strategies. At December 31, 2018 and 2017, the Company has recorded a full valuation allowance.

Reserves are provided for tax benefits for which realization is uncertain. Such benefits are only recognized when the underlying tax position is considered more-likely-than-not to be sustained on examination by a taxing authority, assuming they possess full knowledge of the position and facts. Interest and penalties related to uncertain tax positions are recognized in the provision of income taxes; however, the Company currently has no interest or penalties related to uncertain income tax benefits.

The Tax Cuts and Jobs Act (the TCJA) was enacted on December 22, 2017. The TCJA reduces the U.S. federal corporate tax rate from a top rate of 35% to a flat rate of 21%. The Company continues to monitor for legislative developments, issuance of regulations and technical memorandum to provide further clarification and/or interpretations of the TCJA and will adjust its consolidated financial statements as needed.

Net loss per share

The Company calculates basic and diluted net loss per share attributable to common stockholders in conformity with the two-class method required for participating securities. The Company considers its convertible preferred stock (Preferred Stock) to be participating securities as in the event a dividend is paid on common stock, the holders of Preferred Stock would be entitled to receive dividends on a basis consistent with the common stockholders. Under the two-class method, the net loss attributable to common stockholders is not allocated to the Preferred Stock as the holders of the Preferred Stock do not have a contractual obligation to share in losses.

Under the two-class method, basic net loss per share attributable to common stockholders is computed by dividing the net loss attributable to common stockholders by the weighted average number of shares of common stock.

Segment and geographic information

Operating segments are defined as components of an entity about which separate discrete information is available for evaluation by the chief operating decision maker, or decision-making group, in deciding how to allocate resources and in assessing performance. The Company operates in one segment in the United States. The Company’s chief executive officer, as the chief operating decision-maker, manages and allocates resources to the operations of the Company on a total company basis using consolidated financial information.

Emerging growth company and smaller reporting company status

The Company is an emerging growth company, as defined in the Jumpstart Our Business Startups Act of 2012 (the JOBS Act). Under the JOBS Act, emerging growth companies can delay adopting new or revised accounting standards issued subsequent to the enactment of the JOBS Act, until such time as those standards apply to private companies.

The Company has elected to use this extended transition period for complying with new or revised accounting standards that have different effective dates for public and private companies until the earlier of the date that it is (i) no longer an emerging growth company or (ii) affirmatively and irrevocably opt out of the extended transition period provided in the JOBS Act. As a result, the Company’s consolidated financial statements may

not be comparable to companies that comply with the new or revised accounting pronouncements as of public company effective dates.

The Company will remain an emerging growth company until the earliest of (i) the last day of the Company's first fiscal year (a) following the fifth anniversary of the completion of this offering, (b) in which the Company has total annual gross revenues of at least $1.07 billion, or (c) when the Company is deemed to be a large accelerated filer, which means the market value of the Company's common stock that is held by non-affiliates exceeds $700.0 million as of the prior June 30th and (ii) the date on which the Company has issued more than $1.0 billion in non-convertible debt securities during the prior three-year period.

The Company is also a "smaller reporting company," meaning that in the event of an initial public offering the market value of its stock held by non-affiliates plus the proposed aggregate amount of gross proceeds to the Company as a result of such offering is less than $700 million and its annual revenue is less than $100 million during the most recently completed fiscal year. The Company may continue to be a smaller reporting company as long as either (i) the market value of its stock held by non-affiliates is less than $250 million or (ii) its annual revenue is less than $100 million during the most recently completed fiscal year and the market value of its stock held by non-affiliates is less than $700 million. If the Company is a smaller reporting company at the time it ceases to be an emerging growth company, the Company may continue to rely on exemptions from certain disclosure requirements that are available to smaller reporting companies. Specifically, as a smaller reporting company, the Company may choose to present only the two most recent fiscal years of audited financial statements in its Annual Report on Form 10-K and, similar to emerging growth companies, smaller reporting companies have reduced disclosure obligations regarding executive compensation.

Recently adopted accounting pronouncements

In May 2017, the FASB issued ASU 2017-09, *Compensation—Stock Compensation* (Topic 718): *Scope of Modification Accounting*, which provides guidance about which changes to the terms or conditions of a share-based payment award require an entity to apply modification accounting in Topic 718. The amendments in this ASU should be applied prospectively to an award modified on or after the adoption date. The Company adopted ASU 2017-09 effective January 1, 2018, and the adoption of ASU 2017-09 did not impact the Company's consolidated financial statements or financial statement disclosures.

Recently issued accounting pronouncements

In February 2016, the FASB issued ASU 2016-02, *Leases* (Topic 842), with guidance regarding the accounting for and disclosure of leases. The update requires lessees to recognize all leases, including operating leases, with a term greater than 12 months on the consolidated balance sheet. This update also requires lessees and lessors to disclose key information about their leasing transactions. This guidance will be effective for public companies for annual and interim periods beginning after December 15, 2018. For all other entities, this standard is effective for annual reporting periods beginning after December 15, 2019, and interim periods within annual periods beginning after December 15, 2020. The Company will adopt this standard on January 1, 2020. While the Company expects the implementation of ASU 2016-02 to result in the recognition of right-of-use assets and lease liabilities for leased facilities, the Company is still evaluating the impact that the adoption of ASU 2016-2 will have on its consolidated financial statements.

In July 2017, the FASB issued ASU 2017-11, *Earnings Per Share* (Topic 260), Distinguishing Liabilities from Equity (Topic 480) and Derivatives and Hedging (Topic 815): *I. Accounting for Certain Financial Instruments with Down Round Features; II. Replacement of the Indefinite Deferral for Mandatorily Redeemable Financial Instruments of Certain Nonpublic Entities and Certain Mandatorily Redeemable Noncontrolling Interests with a Scope Exception*. Part I of this update addresses the complexity of accounting for certain financial instruments with down round features. Down round features are features of certain equity-linked instruments (or embedded features) that result in the strike price being reduced on the basis of the pricing of future equity offerings. Current accounting

guidance creates cost and complexity for entities that issue financial instruments (such as warrants and convertible instruments) with down round features that require fair value measurement of the entire instrument or conversion option. Part II of this update addresses the difficulty of navigating Topic 480, Distinguishing Liabilities from Equity, because of the existence of extensive pending content in the FASB Accounting Standards Codification. This pending content is the result of the indefinite deferral of accounting requirements about mandatorily redeemable financial instruments of certain nonpublic entities and certain mandatorily redeemable noncontrolling interests. The amendments in Part II of this update do not have an accounting effect. For public business entities, the amendments in Part I of ASU-2017-11 are effective for fiscal years and interim periods within those years beginning after December 15, 2018. For all other entities, the amendments in Part I of this update are effective for fiscal years beginning after December 15, 2019, and interim periods within fiscal years beginning after December 15, 2020. The Company intends to adopt Part I of this update on January 1, 2020. Early adoption is permitted for all entities, including adoption in an interim period. The Company is currently assessing the potential impact of adopting ASU 2017-11 on its consolidated financial statements and financial statement disclosures.

In August 2018, the FASB issued ASU 2018-13, *"Fair Value Measurement (Topic 820), Disclosure Framework—Changes to the Disclosure Requirements for Fair Value Measurement".* This ASU removed the following disclosure requirements: (1) the amount of and reasons for transfers between Level 1 and Level 2 of the fair value hierarchy; (2) the policy for timing of transfers between levels; and (3) the valuation processes for Level 3 fair value measurements. Additionally, this update added the following disclosure requirements: (1) the changes in unrealized gains and losses for the period included in other comprehensive income and loss for recurring Level 3 fair value measurements held at the end of the reporting period; (2) the range and weighted average of significant unobservable inputs used to develop Level 3 fair value measurements. For certain unobservable inputs, an entity may disclose other quantitative information (such as the median or arithmetic average) in lieu of the weighted average if the entity determines that other quantitative information would be a more reasonable and rational method to reflect the distribution of unobservable inputs used to develop Level 3 fair value measurements. ASU 2018-13 will be effective for all entities, for fiscal years beginning after December 15, 2019 with early adoption permitted. The Company intends to adopt this standard on January 1, 2020 and does not expect that the adoption of this update will have a material impact on its consolidated financial statements.

3. Fair value measurements

There were no assets or liabilities carried at fair value on a recurring basis as of December 31, 2018. As of December 31, 2017, the Company had a $3,000 Simple Agreement for Future Equity (SAFE) obligation, which represents a Level 3 measurement within the fair value hierarchy.

The following tables present information about the Company's financial assets measured at fair value on a recurring basis and indicate the level of the fair value hierarchy utilized to determine such fair values:

	Fair value measurements as of December 31, 2018			
	Level 1	Level 2	Level 3	Total
Cash equivalents:				
Money market funds	$105,399	$ —	$ —	$105,399
Total	$105,399	$ —	$ —	$105,399

	Fair value measurements as of December 31, 2017			
	Level 1	Level 2	Level 3	Total
Cash equivalents:				
Money market funds	$ 1,797	$ —	$ —	$1,797
Total	$ 1,797	$ —	$ —	$1,797
Debt:				
Simple agreement for future equity	$ —	$ —	$ 3,000	$3,000
Total	$ —	$ —	$ 3,000	$3,000

The Company's assets with fair value categorized as Level 1 within the fair value hierarchy include money market funds. Money market funds are publicly traded mutual funds and are presented as cash equivalents on the consolidated balance sheets as of December 31, 2018 and 2017.

The following table presents a roll-forward of the fair value of the SAFE for which fair value is determined by Level 3 inputs:

Balance as of January 1, 2017	$ —
Initial fair value	3,000
Fair value adjustment	—
Balance as of December 31, 2017	3,000
Fair value adjustment	—
Conversion into Preferred Stock, January 2018	(3,000)
Balance as of December 31, 2018	$ —

Fair value of the SAFE on issuance was determined to be equal to the proceeds received. Fair value of the SAFE on conversion into Preferred Stock (see Notes 6 and 8) was determined to be equal to the fair value of the 788,042 Preferred Stock received ($3,000). Given the proximity of the conversion of the SAFE to the financial reporting period end, the fair value of the SAFE as of December 31, 2017 was also determined to be equal to the fair value of the Preferred Stock, of $3,000, received on conversion.

There were no transfers among Level 1, Level 2, or Level 3 categories in the periods presented.

The carrying value of cash, cash equivalents, accounts payable and accrued expenses that are reported on the consolidated balance sheets approximate their fair value due to the short-term nature of these assets and liabilities.

4. Property and equipment, net

Property, and equipment, net consisted of the following:

	As of December 31,	
	2018	2017
Laboratory equipment	$ 1,485	$ 583
Furniture and fixtures	—	20
Leasehold improvements	—	5
Office equipment	70	37
Construction in progress	16	—
	1,571	645
Less accumulated depreciation	(379)	(172)
	$ 1,192	$ 473

Depreciation expense was $214 and $113 for the years ended December 31, 2018 and 2017, respectively.

5. Accrued and other current liabilities

Accrued and other current liabilities consisted of the following:

	As of December 31,	
	2018	2017
Accrued employee compensation costs	$ 901	$ 382
Accrued professional	200	102
Accrued research and development costs	234	70
Accrued other	61	20
	$ 1,396	$ 574

6. Simple agreement for future equity

In October 2017, the Company entered into a simple agreement for future equity (the SAFE) with an investor, receiving $3,000 in exchange for the investor's right to participate in a future equity financing. The SAFE contained a number of conversion and redemption provisions, including settlement upon liquidity or dissolution events. The Company elected the fair value option of accounting for the SAFE (see Note 3). Issue costs of $21 related to the SAFE were recorded as a general and administrative expense for the year ended December 31, 2017 in the accompanying consolidated statements of operations. In January 2018, the investor exercised its rights to convert the SAFE in connection with the Company's equity financing (See Note 8) and exchanged the SAFE for 788,042 shares of Series A-2 convertible Preferred Stock (Series A-2 Preferred).

7. Commitments and contingencies

Operating lease

In December 2015, the Company entered into an agreement to lease its facility under a non-cancellable operating lease that expires December 2019. The lease includes two renewal options, each for five-year terms and at fair market value upon exercise. The lease contains escalating rent clauses which require higher rent payments in future years. The Company expenses rent on a straight-line basis over the term of the lease. The lease was terminated by the Company, effective December 31, 2018, without early termination fees or any additional obligation to the landlord.

In August 2018, the Company entered into an agreement to sublease approximately 23,000 square feet of space for a term of three years. Lease terms are triple net lease commencing at $886 per year, then with three percent annual base rent increases plus operating expenses, real estate taxes, utilities and janitorial fees. The lease commencement date was December 10, 2018.

In December 2018, the Company entered into an agreement to lease 2,485 square feet of space for a term of three years. The lease includes one renewal option for an additional two years. Lease terms commence at $231 per annum, with 2.5% annual base rent increases plus operating expenses, real estate taxes, utilities and janitorial fees. The Company expects to occupy this space in the first half of 2019.

As of December 31, 2018, the future minimum payments for operating leases are as follows:

2019	$1,051
2020	1,149
2021	1,102
2022	71
2023	—
Thereafter	—
	$3,373

Rent expense incurred under operating leases was approximately $326 and $235 for the years ended December 31, 2018 and 2017, respectively.

Consulting Agreement

In October 2014, we entered into a consulting agreement with a member of our board of directors, who is also an employee of Cold Spring Harbor Laboratory (CSHL), to provide consulting services related to scientific research related to the development of antisense-based drugs, therapies, diagnostic and research tools, products, services and intellectual property. We made payments of $100 in each of the years ended December 31, 2018 and 2017, respectively, for such consulting services. The initial term of this agreement was five years and may be extended by the mutual consent of the Company and the board member.

License and research agreements

In July 2015, the Company entered into a worldwide license agreement, or the CSHL Agreement, with CSHL, with respect to Targeted Augmentation of Nuclear Gene Output (TANGO) patents. Under the CSHL Agreement, the Company receives an exclusive (except with respect to certain government rights and non-exclusive licenses), worldwide license under certain patents and applications relating to TANGO. As part of the CSHL Agreement, the Company granted CSHL 164,927 shares of common stock valued based on an independent appraisal at approximately $65. The CSHL Agreement obligates the Company to make additional payments that are contingent upon certain milestones being achieved. The Company is also required to pay royalties, tiered based on the scope of patent coverage for each licensed product, ranging from a low-single digit percentage to a mid-single digit percentage on annual net sales. These royalty obligations apply on a licensed product-by-licensed product and country-by-country basis until the latest of (i) the expiration of the last valid claim of a CSHL patent covering the applicable licensed product or (ii) the expiration of any regulatory exclusivity for the applicable licensed product. In addition, if the Company sublicenses the rights under the CSHL Agreement, the Company is required to pay a low double-digit percentage of the sublicense revenue to CSHL, which may be reduced to a low double-digit or a mid-single digit percentage upon achievement of certain clinical milestones for the applicable licensed product. Finally, the Company is required to pay an annual license maintenance fee of $10, which amount is creditable against any owed royalty or milestone payments. The maximum aggregate potential milestone payments payable total approximately $900. Additionally, certain licenses under the CSHL Agreement require the Company to reimburse CSHL for certain past and ongoing patent related expenses, however there were no expenses related to these reimbursable patent costs during the years ended December 31, 2018 and 2017.

In April 2016, the Company entered into an exclusive, worldwide license agreement with the University of Southampton, or the Southampton Agreement, whereby the Company acquired rights to foundational technologies related to the Company's TANGO technology. Under the Southampton Agreement, the Company receives an exclusive, worldwide license under certain licensed patents and applications relating to TANGO. As part of the Southampton Agreement, the Company paid 55 pounds sterling (approximately $72 as of the date thereof) as an up-front license fee. Under the Southampton Agreement, the Company may be obligated to make additional payments that are contingent upon certain milestones being achieved, as well as royalties on future product sales. These royalty obligations survive until the latest of (i) the expiration of the last valid claim of a licensed patent covering a subject product or (ii) the expiration of any regulatory exclusivity for the subject product in a country. In addition, if the Company sublicenses its rights under the Southampton Agreement, the Company is required to pay a mid-single digit percentage of the sublicense revenue to the University of Southampton. The maximum aggregate potential milestone payments payable by the Company total approximately 400 pounds sterling (approximately $508 as of December 31, 2018). As of December 31, 2018, and 2017, the Company has recorded no liabilities under the Southampton Agreement.

Sponsored research agreement

In December 2017, the Company entered into a sponsored research agreement with the University of Michigan (the University of Michigan Agreement) to fund research conducted at the University of Michigan through November 2018. The budget for the scope of work described in the research agreement was $428. In December 2018, the University of Michigan Agreement was renewed and extended through November 2020 with a budget for the scope of work of $624. The costs incurred by the Company under the University of Michigan Agreement are recorded as research and development expense and expensed in the period in which they are incurred. Research and development spending under the University of Michigan Agreement was $428 and $12 for years ended December 31, 2018 and 2017, respectively.

Litigation

The Company may periodically become subject to legal proceedings and claims arising in connection with ongoing business activities, including claims or disputes related to patents that have been issued or that are pending in the field of research on which it is focused. As of December 31, 2018, the Company had no legal proceedings to which it was a party or to which its property was subject.

8. Convertible preferred stock

As of December 31, 2018, the Company's amended and restated certificate of incorporation authorized the Company to issue 225,584,874 shares of $0.0001 par value convertible preferred stock, 49,540,132 are designated Series A convertible preferred stock (Series A Preferred), 75,777,370 are designated Series A-2 Preferred and 100,267,372 are designated Series B convertible preferred stock (Series B Preferred) (collectively, the Preferred Stock). The following table summarizes the Company's issued and outstanding Preferred Stock:

	Series A Preferred		Series A-2 Preferred		Series B Preferred		Total convertible Preferred	
	Shares	Amount	Shares	Amount	Shares	Amount	Shares	Amount
Balance, January 1, 2017	3,719,866	$ 8,750	—	$ —	—	$ —	3,719,366	$ 8,750
Issuance, net of issuance costs of $25	1,260,802	2,975	—	—	—	—	1,260,802	2,975
Balance, December 31, 2017	4,980,168	11,725	—	—	—	—	4,980,168	11,725
Issuance upon conversion of SAFE	—	—	788,042	3,000	—	—	788,042	3,000
Issuance, net of issuance costs of $120	—	—	6,829,704	25,880	—	—	6,829,704	25,880
Issuance, net of issuance costs of $258	—	—	—	—	10,079,671	89,742	10,079,671	89,742
Balance, December 31, 2018	4,980,168	$ 11,725	7,617,746	$ 28,880	10,079,671	$ 89,742	22,677,585	$ 130,347

The Company's Preferred Stock has the following rights and preferences, privileges and restrictions:

Voting rights

On any matter presented to the stockholders of the Company for their action or consideration at any meeting of stockholders of the Company, each holder of outstanding shares of Preferred Stock shall be entitled to cast the number of votes equal to the number of whole shares of Common Stock into which the shares of Preferred Stock held by such holder are convertible as of the record date for determining stockholders entitled to vote on such matter. Except as provided by law or by the other provisions of the certificate of incorporation of the Company, holders of Preferred Stock shall vote together with the holders of Common Stock as a single class.

For so long as any shares of Preferred Stock remain outstanding, the holders of record of the shares of Preferred Stock, exclusively and as a separate class, shall be entitled to elect two directors of the Company.

Dividends

The holders of shares of Preferred Stock shall be entitled to receive non-cumulative dividends at the rate of eight percent of the original issue price (see below) of such series of such Preferred Stock per annum on such shares of Preferred Stock (subject to appropriate adjustment in the event of any stock dividend, stock split, combination or other similar recapitalization with respect to the Preferred Stock) when, as, and if declared by the Board of Directors (the Preferred Dividend).

The Company shall not declare, pay or set aside any dividends on shares of any other class or series of capital stock of the Company (other than dividends on shares of Common Stock payable in shares of Common Stock) unless the holders of the Preferred Stock then outstanding shall first receive, or simultaneously receive, a dividend on each outstanding share of Preferred Stock in an amount at least equal to the sum of (i) the amount of the Preferred Dividend then accrued on such share of Preferred Stock for the calendar year in which such dividends are being paid hereunder and not previously paid and (ii) (A) in the case of a dividend on common stock or any class or series that is convertible into common stock, that dividend per share of Preferred Stock as would equal the product of (l) the dividend payable on each share of such class or series determined, if applicable, as if all shares of such class or series had been converted into common stock and (2) the number of shares of common stock issuable upon conversion of a share of such Preferred Stock, in each case calculated on the record date for determination of holders entitled to receive such dividend or (B) in the case of a dividend on any class or series that is not convertible into common stock, at a rate per share of Preferred Stock determined by (1) dividing the amount of the dividend payable on each share of such class or series of capital stock by the original issuance price of such class or series of capital stock (subject to appropriate adjustment in the event of any stock dividend, stock split, combination or other similar recapitalization with respect to such class or series) and (2) multiplying such fraction by an amount equal to the original issue price (see below) of such Preferred Stock; provided that, if the Company declares, pays or sets aside, on the same date, a dividend on shares of more than one class or series of capital stock of the Company, the dividend payable to the holders of a series of Preferred Stock shall be calculated based upon the dividend on the class or series of capital stock that would result in the highest dividend to such series of Preferred Stock.

The original issue price shall mean $8.9289 per share of Series B Preferred, subject to appropriate adjustment in the event of any stock dividend, stock split, combination or other similar recapitalization with respect to the Series B Preferred, $2.3794 per share of Series A Preferred, subject to appropriate adjustment in the event of any stock dividend, stock split, combination or other similar recapitalization with respect to the Series A Preferred, and $3.8069 per share of Series A-2 Preferred, subject to appropriate adjustment in the event of any stock dividend, stock split, combination or other similar recapitalization with respect to the Series A-2 Preferred.

Optional conversion rights

Each share of Preferred Stock shall be convertible, at the option of the holder thereof, at any time and from time to time, and without the payment of additional consideration by the holder thereof, into such number of fully paid and non-assessable shares of common stock as is determined by dividing the original issue price of such share of Preferred Stock by the conversion price (as described below) of such share of Preferred Stock in effect at the time of conversion. The conversion price for each share of Preferred Stock shall initially be equal to the original issue price of such share of Preferred Stock. Such initial conversion price, and the rate at which shares of Preferred Stock may be converted into shares of common stock, is subject to adjustment.

Mandatory conversion rights

Upon either (a) an initial public offering valuing the company at least $275,000 and for total offering proceeds not less than $75,000 or (b) the date and time, or the occurrence of an event, specified by vote or written consent of (i) a majority of the then-outstanding shares of Series A Preferred and Series A-2 Preferred, voting as a single class and (b) a majority of the outstanding Series B Preferred Stock voting together as a single class,

then all outstanding shares of Preferred Stock shall automatically be converted into shares of common stock, at the then effective conversion rate.

Liquidation

In the event of (i) any voluntary or involuntary liquidation, dissolution or winding up of the Company or (ii) the merger or consolidation of the Company or a subsidiary relinquishing a majority of the voting power of the capital stock, or the sale, lease, transfer, exclusive license or other disposition of all or substantially all assets of the Company (Deemed Liquidation Event), the holders of shares of Preferred Stock then outstanding shall be entitled to be paid out of the assets of the Company available for distribution to its stockholders before any payment shall be made to the holders of common stock by reason of their ownership thereof, an amount per share equal to the original issue price of such share of Preferred Stock, plus any dividends declared but unpaid thereon. If upon any such liquidation, dissolution or winding up of the Company or Deemed Liquidation Event, the assets of the Company available for distribution to its stockholders shall be insufficient to pay the holders of shares of Preferred Stock the full amount to which they shall be entitled, the holders of shares of Preferred Stock shall share ratably and on a *pari passu* basis in any distribution of the assets available for distribution in proportion to the respective amounts which would otherwise be payable in respect of the shares held by them upon such distribution if all amounts payable on or with respect to such shares were paid in full.

In the event of any voluntary or involuntary liquidation, dissolution or winding up of the Company or Deemed Liquidation Event, after the payment of all preferential amounts required to be paid to the holders of shares of Preferred Stock the remaining assets of the Company available for distribution to its stockholders shall be distributed among the holders of the shares of Preferred Stock and common stock, pro rata based on the number of shares held by each such holder, treating for this purpose all such securities as if they had been converted to common stock pursuant to the terms of the certificate of incorporation of the Company immediately prior to such liquidation, dissolution or winding up of the Company.

Anti-Dilution

Holders of convertible Preferred Stock are afforded certain anti-dilution protection with respect to corporate events such as stock splits and recapitalizations.

Redemption

The Company's convertible Preferred Stock is not redeemable except in the event of any voluntary or involuntary liquidation, dissolution or winding up of the Company or a Deemed Liquidation Event, and only if elected by a majority of the Company's Board of Directors.

9. Common stock

In June 2014, the Company issued 502,638 shares of common stock to its two founders. The shares vest at a rate of 1/3 on the first anniversary date and in 36 equal monthly installments thereafter. Vested shares as of December 31, 2018 and 2017 were 502,638 and 446,791 shares, respectively. In July 2015, the Company issued 164,927 shares of common stock to CSHL in exchange for an exclusive license to a patent (Note 7).

The holders of record of the shares of common stock, exclusively and as a separate class, shall be entitled to elect two directors of the Company. The holders of the common stock and one Preferred Stock investor, voting together as a single class on an as-if-converted basis, shall be entitled to elect two directors and the holders of the common stock and the Preferred Stock, voting together as a single class on an as-if-converted basis, shall be entitled to elect one director.

10. 2014 equity incentive plan

In June 2014, the Company's board of directors and stockholders approved the 2014 Equity Incentive Plan (the 2014 Plan) under which it may grant incentive stock options, non-qualified stock options, restricted stock

awards, unrestricted stock awards, or restricted stock units to purchase up to 679,222 shares of common stock to employees, officers, directors and consultants of the Company. In January 2018, the Company increased the number of shares of common stock reserved for issuance under the 2014 Plan to 4,652,098 shares.

In 2016, the Company granted options to purchase 511,450 shares of common stock to certain of its employees, and directors. The options vest over three to four years and are exercisable at a per share price equal to the fair value of the common stock on the grant date.

In 2017, the Company granted options to purchase 143,487 shares of common stock to certain of its employees and directors. The options vest over four years and are exercisable at a per share price equal to the fair value of the common stock on the grant date.

In 2018, the Company granted options to purchase 2,969,181 shares of common stock to certain of its employees, and directors. The options vest over four years and are exercisable at a per share price equal to the fair value of the common stock on the grant date.

As of December 31, 2018, there were 1,110,145 shares available for future issuance under the 2014 Plan.

As there is not a public market for the Company's common stock, the Company has determined the volatility for options granted in 2017 based on a study of reported data for a guideline group of companies that issued options with substantially similar terms. The risk-free interest rate is based on a zero-coupon United States Treasury instrument with terms consistent with the expected life of the stock options. The Company has not paid and does not anticipate paying cash dividends on shares of common stock; therefore, the expected dividend yield is assumed to be zero.

A summary of stock option activity for awards is presented below:

	Number of shares	Weighted average exercise price	Weighted average remaining contractual life (years)	Aggregate intrinsic value[1]
Outstanding as of December 31, 2016	501,932	$ 0.40	9.1	$ —
Granted	143,487	0.40		
Forfeited or expired	(59,978)	0.40		
Outstanding as of December 31, 2017	585,441	0.40	8.0	—
Granted	2,969,181	1.34		
Exercised	(57,324)	0.44		
Forfeited or expired	(12,670)	0.40		
Outstanding as of December 31, 2018	3,484,628	$ 1.20	8.9	$ 3,436
Exercisable as of December 31, 2018	891,366	$ 0.58	8.1	$ 1,437

(1) The aggregate intrinsic value is calculated as the difference between the exercise price of the underlying options and the estimated fair value of the common stock for the options that were in the money at December 31, 2018 and 2017.

The weighted average grant date fair value per share of stock options granted during the years ended December 31, 2018 and 2017 was $0.79 and $0.25, respectively. The aggregate intrinsic value of stock options exercised during the year ended December 31, 2018 was $100.

The aggregate grant date fair value of stock options granted during the years ended December 31, 2018 and 2017 was approximately $2,335 and $57, respectively.

Stock-based compensation

The Company recorded stock-based compensation expense of $240 and $26 during the years ended December 31, 2018 and 2017, respectively. As of December 31, 2018, there was $1,986 of unrecognized

compensation cost related to unvested stock-based compensation arrangements granted under the 2014 Plan. The compensation is expected to be recognized over a weighted average period of 4 years as of December 31, 2018.

Stock-based compensation expense recorded as research and development and general and administrative expenses in the accompanying consolidated statements of operations is as follows:

	Year ended December 31,	
	2018	2017
Research and development	$ 117	$ 7
General and administrative	123	19
	$ 240	$ 26

The Company uses the Black-Scholes option pricing model to calculate the grant-date fair value of an award. The fair values of the options granted to employees and directors were calculated using the following assumptions for the year ended December 31, 2018:

	Year ended December 31,	
	2018	2017
Risk-free interest rate	2.67%—2.84%	2.27%
Expected dividend yield	0%	0%
Expected life	6.25—6.375 years	6.25—6.375 years
Expected volatility	57%—60%	65%

11. Net loss per share attributable to common stockholders

The following table summarizes the computation of basic and diluted net loss per share attributable to common stockholders of the Company:

	Year ended December 31,	
	2018	2017
Numerator:		
Net loss	$ (12,521)	$ (5,558)
Denominator:		
Weighted-average number of common shares, basic and diluted	709,336	670,090
Net loss per common share attributable to common stockholders, basic and diluted	$ (17.65)	$ (8.29)

The Company's potential dilutive securities, which include Preferred Stock and common stock options, have been excluded from the computation of diluted net loss per share as the effect would be anti-dilutive. Therefore, the weighted average number of common shares outstanding used to calculate both basic and diluted net loss per share attributable to common stockholders is the same. The Company excluded the following potential common shares, presented based on amounts outstanding at period end, from the computation of diluted net loss per share attributable to common stockholders for the period indicated because including them would have had an anti-dilutive effect:

	December 31,	
	2018	2017
Preferred Stock	22,677,585	4,980,168
Outstanding options to purchase common stock	3,484,628	585,441
	26,162,213	5,565,609

Unaudited pro forma net loss per share

The unaudited pro forma basic and diluted weighted average common shares outstanding used in the calculation of unaudited pro forma basic and diluted net loss per share attributable to common stockholders for the year ended December 31, 2018 gives effect to the automatic conversion upon the closing of the proposed initial public offering of all outstanding shares of convertible preferred stock as of December 31, 2018 into 22,677,585 shares of common stock as if the conversion had occurred on the later of January 1, 2018 or the issuance date of the convertible preferred stock.

Unaudited pro forma basic and diluted net loss per share attributable to common stockholders was calculated as follows (in thousands, except share and per share amounts):

	Year ended December 31, 2018 (unaudited)
Numerator:	
Net loss attributable to common stockholders	$ 12,521
Pro forma net loss attributable to common stockholders	$ 12,521
Denominator:	
Weighted average common shares outstanding—basic and diluted	709,336
Pro forma adjustment to reflect assumed automatic conversion of convertible preferred shares upon the closing of the proposed initial public offering	12,075,475
Pro forma weighted average common shares outstanding—basic and diluted	12,784,811
Pro forma net loss per share attributable to common stockholders—basic and diluted	$ (0.98)

12. Income taxes

Among other provisions, the TCJA reduces the historical U.S. federal corporate income tax rate from a top rate of 35% to a newly enacted flat rate of 21% beginning January 1, 2018. At the date the new legislation is enacted, under ASC 740, *Income Taxes*, the Company is required to recognize the effects of the change in tax law and rates on its deferred tax assets and liabilities as a charge to income tax expense. As a result of the above TCJA and the revaluation of deferred tax assets and liabilities at December 31, 2017, the Company recognized a decrease in its deferred tax assets of $1,506. This reduction in the Company's deferred tax assets has been offset by a coinciding reduction in the associated valuation allowance, resulting in no additional tax expense.

A reconciliation of the expected income tax expense (benefit) computed using the federal statutory income tax rate to the Company's effective income tax rate is as follows:

	Year ended December 31,	
	2018	2017
Expected income tax benefit at the federal statutory rate	21.0	34.0
State income taxes, net of federal benefit	6.2	5.0
Non-deductible items	(0.3)	(1.7)
Research and development credit, net	0.9	6.7
Tax rate reductions due to the TCJA	0.0	(27.2)
Other	0.1	0.0
Change in valuation allowance	(27.9)	(16.8)
Total	0.0%	0.0%

The principal components of the Company's deferred tax assets and liabilities consist of the following:

	As of December 31,	
	2018	2017
Deferred tax assets:		
Federal and state net operating loss carryforwards	$ 6,637	$ 3,380
Research and development tax credits	778	654
Other	210	92
Gross deferred tax assets	7,625	4,126
Less: valuation allowance	(7,625)	(4,126)
Net deferred tax assets	$ —	$ —

As of December 31, 2018 and 2017, the Company had federal net operating loss carryforwards of $24,372 and $12,476, respectively, which may be available to reduce future taxable income, and expire at various dates beginning in 2034, for those net operating loss carryforwards generated prior to 2018. Net operating losses generated in 2018 and beyond have no expiration. As of December 31, 2018 and 2017, the Company had state net operating loss carry forwards of $24,030 and $12,293, respectively, which may be available to reduce future taxable income and expire at various dates beginning in 2034. In addition, at December 31, 2018 and 2017, the Company had federal research and development tax credit carryforwards of $443 and $172, respectively, and state research and development tax credit carry forwards of $425 and $241, respectively. Both federal and state research and development tax credit carry forwards may be available to reduce future tax liabilities and expire at various dates beginning in 2030. In accordance with Statement of ASC 740, *Accounting for Income Taxes*, management of the Company has evaluated the positive and negative evidence bearing upon the realizability of its deferred tax assets, which are comprised principally of net operating loss carryforwards. Management has determined that it is more likely than not that the Company will not recognize the benefits of federal and state deferred tax assets and, as a result, a full valuation allowance of $7,625 and $4,126 was established at December 31, 2018 and 2017, respectively. The change in the valuation allowance was an increase of $3,499 and $932 in 2018 and 2017, respectively.

Utilization of the net operating loss carryforwards and research and development tax credit carryforwards may be subject to a substantial annual limitation under Sections 382 and 383 of the Internal Revenue Code of 1986, as amended (the Code) due to ownership changes that have occurred previously or that could occur in the future. These ownership changes may limit the amount of carryforwards that can be utilized annually to offset future taxable income. The Company has not conducted a formal study to assess whether a change of control

has occurred or whether there have been multiple changes of control since inception due to the significant complexity and cost associated with such a study. If the Company has experienced a change of control, as defined for purposes of Section 382 and 383 of the Code, at any time since inception, utilization of the net operating loss carryforwards or research and development tax credit carryforwards may be subject to an annual limitation under Section 382 and 383 of the Code, which is determined by first multiplying the value of the Company's stock at the time of the ownership change by the applicable long-term tax-exempt rate, and then could be subject to additional adjustments, as required. Any limitation may result in expiration of a portion of the net operating loss carryforwards or research and development tax credit carryforwards before utilization.

The Company applies ASC 740 related to accounting for uncertainty in income taxes. The Company's reserves related to income taxes are based on a determination of whether, and how much of, a tax benefit taken by the Company in its tax filings or positions is more likely than not to be realized following resolution of any potential contingencies present related to the tax benefit. At December 31, 2018, and 2017 the Company had no unrecognized tax benefits. Interest and penalty charges, if any, related to unrecognized tax benefits would be classified as income tax expense in the accompanying consolidated statements of operations.

13. Employee benefits

In 2016, the Company established a defined-contribution savings plan under Section 401(k) of the Internal Revenue Code (the 401(k) Plan). The 401(k) Plan covers all employees who meet defined minimum age and service requirements and allows participants to defer a portion of their annual compensation on a pretax basis. The Company is not required to make and has not made any contributions to the 401(k) Plan for the years ended December 31, 2018 and 2017.

14. Subsequent events

The Company has evaluated subsequent events through March 26, 2019, the date the consolidated financial statements were originally issued, and has evaluated for disclosures and subsequent events occurring after such date through June 7, 2019, which is the date these consolidated financial statements were available for reissuance. The Company has concluded that no events or transactions have occurred that require disclosure in the accompanying consolidated financial statements other than the following:

On June 6, 2019, the Company effected a one-for-9.95 reverse split of the Company's outstanding and issued common and convertible preferred stock. All common share, preferred share and per share information and related information included in the accompanying consolidated financial statements have been adjusted retroactively, where applicable, to reflect the reverse split (see note 8).

On June 6, 2019, the Company's stockholders approved the 2019 Equity Incentive Award Plan ("2019 Plan") which will become effective in connection with the effectiveness of the Company's proposed initial public offering. The 2019 Plan provides for the granting of equity-based awards. Upon the effectiveness of the Company's proposed initial public offering, 2,200,000 shares of common stock will become available for future issuance plus any reserved shares not issued or subject to outstanding grants under the 2014 Plan on the effective date of the 2019 Plan, for issuance pursuant to awards granted under the 2019 Plan. The provisions of the 2019 Plan allow for periodic automatic increases for shares reserved under the 2019 Plan.

On June 6, 2019, the Company's stockholders approved the 2019 Employee Stock Purchase Plan ("2019 ESPP") which will become effective upon the effectiveness of the Company's proposed initial public offering. The 2019 ESPP initially provides for the issuance of up to 315,000 shares of common stock to employees. The provisions of the 2019 ESPP provide for automatic periodic increases for shares reserved under the 2019 ESPP.

On June 6, 2019, the Company's stockholders approved an amendment to the Company's Amended and Restated Certificate of Incorporation which will become effective in connection with the effectiveness of the Company's proposed initial public offering, to provide that the authorized capital stock of the Corporation shall consist of 300 million shares of common stock, $0.0001 per share par value, and 10 million shares of undesignated preferred stock, $0.0001 per share par value.

Stoke Therapeutics, Inc.
Consolidated balance sheets
(in thousands, except share and per share amounts)
(unaudited)

	As of March 31, 2019	December 31, 2018
Assets		
Current assets:		
Cash and cash equivalents	$ 98,651	105,399
Prepaid expenses and other current assets	1,344	548
Deferred offering costs	1,085	—
Interest receivable	196	196
Total current assets	101,276	106,143
Restricted cash	262	204
Property and equipment, net	1,396	1,192
Total assets	$ 102,934	$ 107,539
Liabilities and stockholders' equity		
Current liabilities:		
Accounts payable	$ 1,126	1,071
Accrued and other current liabilities	2,217	1,396
Total current liabilities	3,343	2,467
Long term liabilities	10	4
Total liabilities	3,353	2,471
Commitments and contingencies (Note 6)		
Stockholders' equity		
Convertible Preferred Stock, par value of $0.0001 per share; 225,584,874 shares authorized as of March 31, 2019 (unaudited) and December 31, 2018; 22,677,585 shares issued and outstanding as of March 31, 2019 (unaudited) and December 31, 2018; aggregate liquidation preference of $130,850 at March 31, 2019 (unaudited) and December 31, 2018	2	2
Common stock, par value of $0.0001 per share; 278,527,249 shares authorized, 892,223 and 727,413 shares issued and outstanding as of March 31, 2019 (unaudited) and December 31, 2018, respectively	—	—
Additional paid-in capital	131,031	130,776
Accumulated deficit	(31,452)	(25,710)
Total stockholders' equity	99,581	105,068
Total liabilities and stockholders' equity	$ 102,934	$ 107,539

The accompanying notes are an integral part of these unaudited consolidated financial statements.

Stoke Therapeutics, Inc.
Consolidated statements of operations
(in thousands, except share and per share amounts)
(unaudited)

	Three months ended March 31,	
	2019	**2018**
Revenue	$ —	$ —
Operating expenses:		
Research and development	4,133	1,252
General and administrative	2,189	660
Total operating expenses	6,322	1,912
Loss from operations	(6,322)	(1,912)
Other income (expense):		
Interest income	580	—
Other expense, net	—	—
Total other income (expense)	580	—
Net loss	$ (5,742)	$ (1,912)
Net loss per share attributable to common stockholders, basic and diluted	$ (6.89)	$ (2.78)
Weighted-average common shares outstanding, basic and diluted	833,469	686,985
Pro forma net loss per share, basic and diluted (Notes 2 and 9)	$ (0.24)	
Weighted-average shares used in computing pro forma net loss per share, basic and diluted (Notes 2 and 9)	23,511,054	

The accompanying notes are an integral part of these unaudited consolidated financial statements.

Stoke Therapeutics, Inc.
Consolidated statements of stockholders' equity
(in thousands, except share and per share amounts)
(unaudited)

	Convertible Preferred Stock		Common Stock				
	Shares	Amount	Shares	Amount	Additional paid-in capital	Accumulated deficit	Stockholders' equity
Balance as of December 31, 2018	22,677,585	$ 2	727,413	$ —	$ 130,776	$ (25,710)	$ 105,068
Stock-based compensation	—	—	—	—	181	—	181
Issuance of common stock	—	—	164,810	—	74	—	74
Net loss	—	—	—	—	—	(5,742)	(5,742)
Balance as of March 31, 2019	22,677,585	$ 2	892,223	$ —	$ 131,031	$ (31,452)	$ 99,581
Balance as of December 31, 2017	4,980,168	—	670,090	—	11,897	(13,189)	(1,292)
Stock-based compensation	—	—	—	—	4	—	4
Issuance of common stock	—	—	21,119	—	8	—	8
Issuance of convertible Preferred Stock, net of issuance costs of $88	4,071,554	1	—	—	15,411	—	15,412
Net loss	—	—	—	—	—	(1,912)	(1,912)
Balance as of March 31, 2018	9,051,722	$ 1	691,209	$ —	$ 27,320	$ (15,101)	$ 12,220

The accompanying notes are an integral part of these unaudited consolidated financial statements.

Stoke Therapeutics, Inc.
Consolidated statements of cash flows
(in thousands)
(unaudited)

	Three months ended March 31,	
	2019	**2018**
Cash flows from operating activities:		
Net loss	$ (5,742)	$ (1,912)
Adjustments to reconcile net loss to net cash used in operating activities:		
Depreciation	80	32
Stock-based compensation	181	4
Loss on disposal of property and equipment	3	—
Changes in assets and liabilities:		
Prepaid expenses and other current assets	(796)	61
Accounts payable and accrued liabilities	(265)	(173)
Deferred rent	7	(1)
Net cash used in operating activities	(6,532)	(1,989)
Cash flows from investing activities:		
Purchases of property and equipment	(92)	(26)
Proceeds from sale of property and equipment	2	—
Net cash used in investing activities	(90)	(26)
Cash flows from financing activities:		
Proceeds from issuance of convertible Preferred Stock	—	12,412
Proceeds from the issuance of common stock	74	8
Deferred offering costs	(141)	—
Other	(1)	(1)
Net cash (used in) provided by financing activities	(68)	12,419
Net (decrease) increase in cash, cash equivalents and restricted cash	(6,690)	10,404
Cash, cash equivalents and restricted cash—beginning of period	105,603	1,853
Cash, cash equivalents and restricted cash—end of Period	$ 98,913	$ 12,257
Supplemental disclosure of non-cash investing and financing activities:		
Property and equipment included in accrued expenses and accounts payable	$ 198	$ 38
Deferred offering costs not yet paid	$ 944	$ —
Issuance of convertible Preferred Stock in exchange for simple agreement for future equity	$ —	$ 3,000

The accompanying notes are an integral part of these unaudited consolidated financial statements.

Stoke Therapeutics, Inc. and subsidiaries
Notes to consolidated financial statements—(unaudited)
(in thousands, except share and per share amounts)

1. Nature of the business and basis of presentation

Organization

Stoke Therapeutics, Inc. (the Company) was founded in June 2014 and was incorporated under the laws of the State of Delaware. The Company is an early-stage biopharmaceutical company pioneering a new way to treat the underlying causes of severe genetic diseases by precisely upregulating protein expression.

On June 6, 2019, the Company effected a 9.95-for-one reverse split of the Company's issued and outstanding common and convertible preferred stock. Upon the effectiveness of the reverse stock split, (i) all shares of outstanding common stock and convertible preferred stock were adjusted; (ii) the number of shares of common stock for which each outstanding option to purchase common stock is exercisable were adjusted; and (iii) the exercise price of each outstanding option to purchase common stock were adjusted. All of the outstanding common stock share numbers (including shares of common stock subject to the Company's options and as converted for the outstanding convertible preferred stock shares), share prices, exercise prices and per share amounts contained in the interim consolidated financial statements have been retroactively adjusted in the interim consolidated financial statements to reflect this reverse stock split for all periods presented. The par value per share and the authorized number of shares of common stock and convertible preferred stock were not adjusted as a result of the reverse stock split.

Uncertainties

The Company is subject to risks and uncertainties common to early-stage companies in the biotechnology industry including, but not limited to, development by competitors of new technological innovations, dependence on key personnel, protection of proprietary technology, compliance with government regulations and ability to secure additional capital to fund operations. Product candidates currently under development will require significant additional research and development efforts, including extensive preclinical and clinical testing and regulatory approval prior to commercialization. These efforts require significant amounts of additional capital, adequate personnel and infrastructure and extensive compliance-reporting capabilities. Even if the Company's product development efforts are successful, it is uncertain when, if ever, the Company will realize significant revenue from product sales.

Liquidity

The Company expects that its operating losses and negative cash flows will continue for the foreseeable future. As of the issuance date of these unaudited consolidated financial statements as of and for the three months ended March 31, 2019, the Company expects that its cash and cash equivalents will be sufficient to fund its operating expenses and capital expenditure requirements through at least twelve months from the issuance date of its consolidated financial statements. The future viability of the Company beyond that date is dependent on its ability to raise additional capital to finance its operations.

The Company is seeking to complete an initial public offering ("IPO") of its common stock. Upon the closing of a qualified IPO (as defined in the Company's Certificate of Incorporation, as amended and restated) on specified terms, all of the Company's outstanding convertible preferred stock will automatically convert into shares of common stock. In the event the Company does not complete an IPO, the Company expects to seek additional funding through private equity financings, debt financings, other collaborations, strategic alliances and licensing arrangements. The Company may not be able to obtain financing on acceptable terms, or at all, and the Company may not be able to enter into strategic alliances or other arrangements on favorable terms, or at

all. The terms of any financing may adversely affect the holdings or the rights of the Company's stockholders. If the Company is unable to obtain funding, the Company could be required to delay, reduce or eliminate research and development programs, product portfolio expansion or future commercialization efforts, which could adversely affect its business prospects.

2. Summary of significant accounting policies and recent accounting pronouncements

Basis of presentation and consolidation

The accompanying unaudited consolidated financial statements have been prepared in accordance with generally accepted accounting principles (GAAP), and include the accounts of Stoke Therapeutics, Inc. and its wholly-owned subsidiary. All intercompany transactions between and among its consolidated subsidiary have been eliminated.

Unaudited interim financial information

The accompanying consolidated balance sheet as of March 31, 2019 and the consolidated statements of operations, stockholders' equity and cash flows for the three months ended March 31, 2019 and 2018 are unaudited. The unaudited interim consolidated financial statements have been prepared on the same basis as the audited annual consolidated financial statements and, in the opinion of management, reflect all adjustments, which include only normal recurring adjustments, necessary for the fair statement of the Company's financial position as of March 31, 2019 and the results of its operations and its cash flows for the three months ended March 31, 2019 and 2018. The financial data and other information disclosed in these notes related to the three months ended March 31, 2019 and 2018 are also unaudited. The results for the three months ended March 31, 2019 are not necessarily indicative of results to be expected for the year ending December 31, 2019, any other interim periods, or any future year or period. The consolidated balance sheet as of December 31, 2018 included herein was derived from the audited consolidated financial statements as of that date. These unaudited consolidated financial statements should be read in conjunction with the Company's audited consolidated financial statements included elsewhere in this prospectus.

Unaudited pro forma information

The accompanying unaudited pro forma consolidated balance sheet as of March 31, 2019 has been prepared to give effect to the automatic conversion of all outstanding shares of convertible preferred stock into 22,677,585 shares of common stock as if the Company's proposed initial public offering had occurred on March 31, 2019.

In the accompanying consolidated statements of operations, the unaudited pro forma basic and diluted net loss per share attributable to common stockholders for the three months ended March 31, 2019 has been prepared to give effect to the automatic conversion of all outstanding shares of convertible preferred stock upon the closing of the proposed initial public offering into 22,677,585 shares of common stock as if the conversion had occurred on the later of January 1, 2019 or the issuance date of the convertible preferred stock.

Use of estimates

The preparation of unaudited consolidated financial statements in conformity with GAAP requires management to make estimates and assumptions that affect the reported amounts of assets, liabilities, equity, expenses and disclosure of contingent assets and liabilities. Estimates are periodically reviewed in light of changes in circumstances, facts and experience. Actual results could differ from our estimates.

Cash and cash equivalents

The Company considers all highly liquid investments with an original maturity of three months or less at the date of purchase to be cash equivalents. The Company deposits its cash in checking, sweep account and money market accounts.

Restricted cash

At March 31, 2019, restricted cash consisted of money market accounts collateralizing letters of credit issued as a security deposits in connection with the Company's lease of its corporate facilities.

The following table reconciles cash and cash equivalents and restricted cash per the consolidated balance sheet to the statement of cash flows:

	As of March 31,	
	2019	**2018**
Cash and cash equivalents	$98,651	12,202
Restricted cash	262	55
	$98,913	$12,257

Fair value of financial instruments

Accounting Standards Codification (ASC) Topic 820, *Fair Value Measurement* (ASC 820), establishes a fair value hierarchy for instruments measured at fair value that distinguishes between assumptions based on market data (observable inputs) and the Company's own assumptions (unobservable inputs). Observable inputs are those that market participants would use in pricing the asset or liability based on market data obtained from sources independent of the Company. Unobservable inputs are inputs that reflect the Company's assumptions about the inputs that market participants would use in pricing the asset or liability and are developed based on the best information available in the circumstances. ASC 820 identifies fair value as the exchange price, or exit price, representing the amount that would be received to sell an asset or paid to transfer a liability in an orderly transaction between market participants. As a basis for considering market participant assumptions in fair value measurements, ASC 820 establishes a three-tier value hierarchy that distinguishes between the following:

Level 1—Quoted market prices in active markets for identical assets or liabilities.

Level 2—Inputs other than Level 1 inputs that are either directly or indirectly observable, such as quoted market prices, interest rates and yield curves.

Level 3—Unobservable inputs developed using estimates of assumptions developed by the Company, which reflect those that a market participant would use.

To the extent the valuation is based on models or inputs that are less observable or unobservable in the market, the determination of fair values requires more judgment. Accordingly, the degree of judgment exercised by the Company in determining fair value is greatest for instruments categorized as Level 3. A financial instrument's level within the fair value hierarchy is based on the lowest level of any input that is significant to the fair value measurement.

Deferred offering costs

The Company capitalizes certain legal, professional accounting and other third-party fees that are directly associated with in-process equity financings as deferred offering costs until such financings are consummated. After consummation of the equity financing, these costs are recorded in stockholders' equity (deficit) as a

reduction of additional paid-in capital generated as a result of the offering. Should the in-process equity financing be abandoned the deferred offering costs will be expensed immediately as a charge to operating expenses in the consolidated statements of operations.

Net loss per share

The Company calculates basic and diluted net loss per share attributable to common stockholders in conformity with the two-class method required for participating securities. The Company considers its convertible preferred stock (Preferred Stock) to be participating securities as in the event a dividend is paid on common stock, the holders of Preferred Stock would be entitled to receive dividends on a basis consistent with the common stockholders. Under the two-class method, the net loss attributable to common stockholders is not allocated to the Preferred Stock as the holders of the Preferred Stock do not have a contractual obligation to share in losses.

Under the two-class method, basic net loss per share attributable to common stockholders is computed by dividing the net loss attributable to common stockholders by the weighted average number of shares of common stock.

Emerging growth company and smaller reporting company status

The Company is an emerging growth company, as defined in the Jumpstart Our Business Startups Act of 2012 (the JOBS Act). Under the JOBS Act, emerging growth companies can delay adopting new or revised accounting standards issued subsequent to the enactment of the JOBS Act, until such time as those standards apply to private companies.

The Company has elected to use this extended transition period for complying with new or revised accounting standards that have different effective dates for public and private companies until the earlier of the date that it is (i) no longer an emerging growth company or (ii) affirmatively and irrevocably opt out of the extended transition period provided in the JOBS Act. As a result, the Company's unaudited consolidated financial statements may not be comparable to companies that comply with the new or revised accounting pronouncements as of public company effective dates.

The Company will remain an emerging growth company until the earliest of (i) the last day of the Company's first fiscal year (a) following the fifth anniversary of the completion of this offering, (b) in which the Company has total annual gross revenues of at least $1.07 billion, or (c) when the Company is deemed to be a large accelerated filer, which means the market value of the Company's common stock that is held by non-affiliates exceeds $700.0 million as of the prior June 30th and (ii) the date on which the Company has issued more than $1.0 billion in non-convertible debt securities during the prior three-year period.

The Company is also a "smaller reporting company," meaning that in the event of an initial public offering the market value of its stock held by non-affiliates plus the proposed aggregate amount of gross proceeds to the Company as a result of such offering is less than $700 million and its annual revenue is less than $100 million during the most recently completed fiscal year. The Company may continue to be a smaller reporting company as long as either (i) the market value of its stock held by non-affiliates is less than $250 million or (ii) its annual revenue is less than $100 million during the most recently completed fiscal year and the market value of its stock held by non-affiliates is less than $700 million. If the Company is a smaller reporting company at the time it ceases to be an emerging growth company, the Company may continue to rely on exemptions from certain disclosure requirements that are available to smaller reporting companies. Specifically, as a smaller reporting company, the Company may choose to present only the two most recent fiscal years of audited financial statements in its Annual Report on Form 10-K and, similar to emerging growth companies, smaller reporting companies have reduced disclosure obligations regarding executive compensation.

Recently issued accounting pronouncements

In February 2016, the FASB issued ASU 2016-02, *Leases* (Topic 842), with guidance regarding the accounting for and disclosure of leases. The update requires lessees to recognize all leases, including operating leases, with a term greater than 12 months on the consolidated balance sheet. This update also requires lessees and lessors to disclose key information about their leasing transactions. This guidance will be effective for public companies for annual and interim periods beginning after December 15, 2018. For all other entities, this standard is effective for annual reporting periods beginning after December 15, 2019, and interim periods within annual periods beginning after December 15, 2020. The Company will adopt this standard on January 1, 2020. While the Company expects the implementation of ASU 2016-02 to result in the recognition of right-of-use assets and lease liabilities for leased facilities, the Company is still evaluating the impact that the adoption of ASU 2016-2 will have on its consolidated financial statements.

In July 2017, the FASB issued ASU 2017-11, *Earnings Per Share* (Topic 260), Distinguishing Liabilities from Equity (Topic 480) and Derivatives and Hedging (Topic 815): *I. Accounting for Certain Financial Instruments with Down Round Features; II. Replacement of the Indefinite Deferral for Mandatorily Redeemable Financial Instruments of Certain Nonpublic Entities and Certain Mandatorily Redeemable Noncontrolling Interests with a Scope Exception*. Part I of this update addresses the complexity of accounting for certain financial instruments with down round features. Down round features are features of certain equity-linked instruments (or embedded features) that result in the strike price being reduced on the basis of the pricing of future equity offerings. Current accounting guidance creates cost and complexity for entities that issue financial instruments (such as warrants and convertible instruments) with down round features that require fair value measurement of the entire instrument or conversion option. Part II of this update addresses the difficulty of navigating Topic 480, Distinguishing Liabilities from Equity, because of the existence of extensive pending content in the FASB Accounting Standards Codification. This pending content is the result of the indefinite deferral of accounting requirements about mandatorily redeemable financial instruments of certain nonpublic entities and certain mandatorily redeemable noncontrolling interests. The amendments in Part II of this update do not have an accounting effect. For public business entities, the amendments in Part I of ASU-2017-11 are effective for fiscal years and interim periods within those years beginning after December 15, 2018. For all other entities, the amendments in Part I of this update are effective for fiscal years beginning after December 15, 2019, and interim periods within fiscal years beginning after December 15, 2020. The Company intends to adopt Part I of this update on January 1, 2020. Early adoption is permitted for all entities, including adoption in an interim period. The Company is currently assessing the potential impact of adopting ASU 2017-11 on its consolidated financial statements and financial statement disclosures.

In August 2018, the FASB issued ASU 2018-13, *"Fair Value Measurement (Topic 820), Disclosure Framework—Changes to the Disclosure Requirements for Fair Value Measurement".* This ASU removed the following disclosure requirements: (1) the amount of and reasons for transfers between Level 1 and Level 2 of the fair value hierarchy; (2) the policy for timing of transfers between levels; and (3) the valuation processes for Level 3 fair value measurements. Additionally, this update added the following disclosure requirements: (1) the changes in unrealized gains and losses for the period included in other comprehensive income and loss for recurring Level 3 fair value measurements held at the end of the reporting period; (2) the range and weighted average of significant unobservable inputs used to develop Level 3 fair value measurements. For certain unobservable inputs, an entity may disclose other quantitative information (such as the median or arithmetic average) in lieu of the weighted average if the entity determines that other quantitative information would be a more reasonable and rational method to reflect the distribution of unobservable inputs used to develop Level 3 fair value measurements. ASU 2018-13 will be effective for all entities, for fiscal years beginning after December 15, 2019 with early adoption permitted. The Company intends to adopt this standard on January 1, 2020 and does not expect that the adoption of this update will have a material impact on its consolidated financial statements.

3. Fair value measurements

There were no assets or liabilities carried at fair value on a recurring basis as of March 31, 2019 and as of December 31, 2018.

The following tables present information about the Company's financial assets measured at fair value on a recurring basis and indicate the level of the fair value hierarchy utilized to determine such fair values:

	Fair value measurements as of March 31, 2019			
	Level 1	Level 2	Level 3	Total
Cash equivalents:				
Money market funds	$98,651	$ —	$ —	$98,651
Total	$98,651	$ —	$ —	$98,651

	Fair value measurements as of December 31, 2018			
	Level 1	Level 2	Level 3	Total
Cash equivalents:				
Money market funds	$105,399	$ —	$ —	$105,399
Total	$105,399	$ —	$ —	$105,399

The Company's assets with fair value categorized as Level 1 within the fair value hierarchy include money market funds. Money market funds are publicly traded mutual funds and are presented as cash equivalents on the consolidated balance sheets as of March 31, 2019 and December 31, 2018.

There were no transfers among Level 1, Level 2, or Level 3 categories in the periods presented.

The carrying value of cash, cash equivalents, accounts payable and accrued expenses that are reported on the consolidated balance sheets approximate their fair value due to the short-term nature of these assets and liabilities.

4. Accrued and other current liabilities

Accrued and other current liabilities consisted of the following:

	March 31, 2019	December 31, 2018
Accrued employee compensation costs	$ 394	$ 901
Accrued professional	1,145	200
Accrued research and development costs	660	234
Accrued other	18	61
	$ 2,217	$ 1,396

5. Simple agreement for future equity

In October 2017, the Company entered into a simple agreement for future equity (the SAFE) with an investor, receiving $3,000 in exchange for the investor's right to participate in a future equity financing. The SAFE contained a number of conversion and redemption provisions, including settlement upon liquidity or dissolution events. The Company elected the fair value option of accounting for the SAFE. In January 2018, the investor exercised its rights to convert the SAFE in connection with the Company's equity financing (See Note 7) and exchanged the SAFE for 788,042 shares of Series A-2 convertible Preferred Stock (Series A-2 Preferred).

6. Commitments and contingencies

Operating lease

In August 2018, the Company entered into an agreement to sublease approximately 23,000 square feet of space for a term of three years. Lease terms are triple net lease commencing at $886 per year, then with three percent annual base rent increases plus operating expenses, real estate taxes, utilities and janitorial fees. The lease commencement date was December 10, 2018.

In December 2018, the Company entered into an agreement to lease 2,485 square feet of space for a term of three years. The lease includes one renewal option for an additional two years. Lease terms commence at $231 per annum, with 2.5% annual base rent increases plus operating expenses, real estate taxes, utilities and janitorial fees. The Company expects to occupy this space in May 2019.

As of March 31, 2019, the future minimum payments for operating leases are as follows:

April 1, 2019 to December 31, 2019	$ 829
2020	1,149
2021	1,102
2022	71
Thereafter	—
	$3,151

Rent expense incurred under operating leases was approximately $240 and $71 for the three months ended March 31, 2019 and 2018, respectively.

Consulting agreement

In October 2014, we entered into a consulting agreement with a member of our board of directors, who is also an employee of Cold Spring Harbor Laboratory (CSHL), to provide consulting services related to scientific research related to the development of antisense-based drugs, therapies, diagnostic and research tools, products, services and intellectual property. We made payments of $25 in each of the three month periods ended March 31, 2019 and 2018, respectively, for such consulting services. The initial term of this agreement was five years and may be extended by the mutual consent of the Company and the board member.

License and research agreements

In July 2015, the Company entered into a worldwide license agreement, or the CSHL Agreement, with CSHL, with respect to Targeted Augmentation of Nuclear Gene Output (TANGO) patents. Under the CSHL Agreement, the Company receives an exclusive (except with respect to certain government rights and non-exclusive licenses), worldwide license under certain patents and applications relating to TANGO. As part of the CSHL Agreement, the Company granted CSHL 164,927 shares of common stock valued based on an independent appraisal at approximately $65. The CSHL Agreement obligates the Company to make additional payments that are contingent upon certain milestones being achieved. The Company is also required to pay royalties, tiered based on the scope of patent coverage for each licensed product, ranging from a low-single digit percentage to a mid-single digit percentage on annual net sales. These royalty obligations apply on a licensed product-by-licensed product and country-by-country basis until the latest of (i) the expiration of the last valid claim of a CSHL patent covering the applicable licensed product or (ii) the expiration of any regulatory exclusivity for the applicable licensed product. In addition, if the Company sublicenses the rights under the CSHL Agreement, the Company is required to pay a low double-digit percentage of the sublicense revenue to CSHL, which may be reduced to a low double-digit or a mid-single digit percentage upon achievement of certain

clinical milestones for the applicable licensed product. Finally, the Company is required to pay an annual license maintenance fee of $10, which amount is creditable against any owed royalty or milestone payments. The maximum aggregate potential milestone payments payable total approximately $900. Additionally, certain licenses under the CSHL Agreement require the Company to reimburse CSHL for certain past and ongoing patent related expenses, however there were no expenses related to these reimbursable patent costs during the three months ended March 31, 2019 and 2018.

In April 2016, the Company entered into an exclusive, worldwide license agreement with the University of Southampton, or the Southampton Agreement, whereby the Company acquired rights to foundational technologies related to the Company's TANGO technology. Under the Southampton Agreement, the Company receives an exclusive, worldwide license under certain licensed patents and applications relating to TANGO. As part of the Southampton Agreement, the Company paid 55 pounds sterling (approximately $72 as of the date thereof) as an up-front license fee. Under the Southampton Agreement, the Company may be obligated to make additional payments that are contingent upon certain milestones being achieved, as well as royalties on future product sales. These royalty obligations survive until the latest of (i) the expiration of the last valid claim of a licensed patent covering a subject product or (ii) the expiration of any regulatory exclusivity for the subject product in a country. In addition, if the Company sublicenses its rights under the Southampton Agreement, the Company is required to pay a mid-single digit percentage of the sublicense revenue to the University of Southampton. The maximum aggregate potential milestone payments payable by the Company total approximately 400 pounds sterling (approximately $518 as of March 31, 2019). As of March 31, 2019, and December 31, 2018, the Company has recorded no liabilities under the Southampton Agreement.

Sponsored research agreement

In December 2017, the Company entered into a sponsored research agreement with the University of Michigan (the University of Michigan Agreement) to fund research conducted at the University of Michigan through November 2018. The budget for the scope of work described in the research agreement was $428. In December 2018, the University of Michigan Agreement was renewed and extended through November 2020 with a budget for the scope of work of $624. The costs incurred by the Company under the University of Michigan Agreement are recorded as research and development expense and expensed in the period in which they are incurred. Research and development spending under the University of Michigan Agreement was $74 and $72 for the three months ended March 31, 2019 and 2018, respectively.

Litigation

The Company may periodically become subject to legal proceedings and claims arising in connection with ongoing business activities, including claims or disputes related to patents that have been issued or that are pending in the field of research on which it is focused. As of March 31, 2019, the Company had no legal proceedings to which it was a party or to which its property was subject.

7. Convertible preferred stock

As of March 31, 2019, and December 31, 2018, the Company's amended and restated certificate of incorporation authorized the Company to issue 225,584,874 shares of $0.0001 par value convertible preferred stock, 49,540,132 are designated Series A convertible preferred stock (Series A Preferred), 75,777,370 are designated Series A-2 Preferred and 100,267,372 are designated Series B convertible preferred stock (Series B Preferred) (collectively, the Preferred Stock). The following table summarizes the Company's issued and outstanding Preferred Stock:

	Series A Preferred		Series A-2 Preferred		Series B Preferred		Total convertible Preferred	
	Shares	Amount	Shares	Amount	Shares	Amount	Shares	Amount
Balance, March 31, 2019	4,980,168	$ 11,725	7,617,746	$ 28,880	10,079,671	$ 89,742	22,677,585	$ 130,347
Balance, December 31, 2018	4,980,168	$ 11,725	7,617,746	$ 28,880	10,079,671	$ 89,742	22,677,585	$ 130,347

8. 2014 equity incentive plan

In June 2014, the Company's board of directors and stockholders approved the 2014 Equity Incentive Plan (the 2014 Plan) under which it may grant incentive stock options, non-qualified stock options, restricted stock awards, unrestricted stock awards, or restricted stock units to purchase up to 679,222 shares of common stock to employees, officers, directors and consultants of the Company. In January 2018, the Company increased the number of shares of common stock reserved for issuance under the 2014 Plan to 4,652,098 shares.

As of March 31, 2019, there were 395,278 shares available for future issuance under the 2014 Plan.

During the three months ended March 31, 2019, the Company granted options to purchase 714,829 shares of common stock to certain of its employees, and directors. The options vest over four years and are exercisable at a per share price equal to the fair value of the common stock on the grant date.

As there is not a public market for the Company's common stock, the Company has determined the volatility for options granted in 2019 based on a study of reported data for a guideline group of companies that issued options with substantially similar terms. The risk-free interest rate is based on a zero-coupon United States Treasury instrument with terms consistent with the expected life of the stock options. The Company has not paid and does not anticipate paying cash dividends on shares of common stock; therefore, the expected dividend yield is assumed to be zero.

A summary of stock option activity for awards is presented below:

	Number of shares	Weighted average exercise price	Weighted average remaining contractual life (years)	Aggregate intrinsic value[1]
Outstanding as of December 31, 2018	3,484,628	$ 1.20	8.9	$ 3,436
Granted	714,829	3.49	—	—
Exercised	(164,810)	0.46	—	—
Forfeited or expired	—	—	—	—
Outstanding as of March 31, 2019	4,034,647	$ 1.64	9.2	$ 11,453
Exercisable as of March 31, 2019	957,629	$ 0.74	8.4	$ 3,575

(1) The aggregate intrinsic value is calculated as the difference between the exercise price of the underlying options and the estimated fair value of the common stock for the options that were in the money at March 31, 2019.

The weighted average grant date fair value per share of stock options granted during the three months ended March 31, 2019 was $2.05. The aggregate intrinsic value of stock options exercised during the three months ended March 31, 2019 was $682.

The aggregate grant date fair value of stock options granted during the three months ended March 31, 2019 was approximately $2,493.

Stock-based compensation

The Company recorded stock-based compensation expense of $181 and $4 during the three months ended March 31, 2019 and 2018, respectively. As of March 31, 2019, there was $3,268 of unrecognized compensation cost related to unvested stock-based compensation arrangements granted under the 2014 Plan. The compensation is expected to be recognized over a weighted average period of 4 years as of March 31, 2019.

Stock-based compensation expense recorded as research and development and general and administrative expenses in the accompanying consolidated statements of operations is as follows:

	Three months ended March 31,	
	2019	2018
Research and development	$ 74	$ —
General and administrative	107	4
	$ 181	$ 4

The Company uses the Black-Scholes option pricing model to calculate the grant-date fair value of an award. The fair values of the options granted to employees and directors were calculated using the following assumptions for the three months ended March 31, 2019 and 2018:

	Three months ended March 31,	
	2019	2018
Risk-free interest rate	2.32-2.81%	—
Expected dividend yield	0%	—
Expected life	6.375 years	—
Expected volatility	57-61%	—

9. Net loss per share attributable to common stockholders

The following table summarizes the computation of basic and diluted net loss per share attributable to common stockholders of the Company:

	Three months ended March 31,	
	2019	2018
Numerator:		
Net loss	$ (5,742)	$ (1,912)
Denominator:		
Weighted-average number of common shares, basic and diluted	833,469	686,985
Net loss per common share attributable to common stockholders, basic and diluted	$ (6.89)	$ (2.78)

The Company's potential dilutive securities, which include Preferred Stock and common stock options, have been excluded from the computation of diluted net loss per share as the effect would be anti-dilutive.

Therefore, the weighted average number of common shares outstanding used to calculate both basic and diluted net loss per share attributable to common stockholders is the same. The Company excluded the following potential common shares, presented based on amounts outstanding at period end, from the computation of diluted net loss per share attributable to common stockholders for the period indicated because including them would have had an anti-dilutive effect:

	March 31,	
	2019	2018
Preferred Stock	22,677,585	9,051,722
Outstanding options to purchase common stock	4,034,647	557,865
	26,712,232	9,609,587

Unaudited pro forma net loss per share

The unaudited pro forma basic and diluted weighted average common shares outstanding used in the calculation of unaudited pro forma basic and diluted net loss per share attributable to common stockholders for the three months ended March 31, 2019 gives effect to the automatic conversion upon the closing of the proposed initial public offering of all outstanding shares of convertible preferred stock as of March 31, 2019 into 22,677,585 shares of common stock as if the conversion had occurred on the later of January 1, 2019 or the issuance date of the convertible preferred stock.

Unaudited pro forma basic and diluted net loss per share attributable to common stockholders was calculated as follows (in thousands, except share and per share amounts):

		Three months ended March 31, 2019 (unaudited)
Numerator:		
Net loss attributable to common stockholders	$	5,742
Pro forma net loss attributable to common stockholders	$	5,742
Denominator:		
Weighted average common shares outstanding—basic and diluted	$	833,469
Pro forma adjustment to reflect assumed automatic conversion of convertible preferred shares upon the closing of the proposed initial public offering		22,677,585
Pro forma weighted average common shares outstanding—basic and diluted		23,511,054
Pro forma net loss per share attributable to common stockholders—basic and diluted	$	(0.24)

10. Income taxes

The company did not record an income tax benefit in its consolidated statement of operations for the three months ended March 31, 2019 and 2018 as it is more likely than not that the Company will not recognize the federal and state deferred tax benefits generated by its losses. The Company had net deferred tax assets and liabilities of $7,625 at December 31, 2018. The Company has provided a valuation allowance for the full amount of its net deferred tax assets and liabilities as of March 31, 2019 and December 31, 2018, as management has determined it is more likely than not that any future benefit from deductible temporary differences and net operating loss and tax credit carryforwards would not be realized.

The Company has not recorded any amounts for unrecognized tax benefits as of March 31, 2019 or December 31, 2018.

11. Employee benefits

In 2016, the Company established a defined-contribution savings plan under Section 401(k) of the Internal Revenue Code (the 401(k) Plan). The 401(k) Plan covers all employees who meet defined minimum age and service requirements and allows participants to defer a portion of their annual compensation on a pretax basis. The Company is not required to make and has not made any contributions to the 401(k) Plan for the three months ended March 31, 2019 and 2018.

12. Subsequent events

The Company has evaluated subsequent events through May 24, 2019, the date that these interim consolidated financial statements were originally issued and subsequent events occurring after such date through June 7, 2019, which is the date these interim consolidated financial statements were available for reissuance. The Company has concluded that no events or transactions have occurred that require disclosure in the accompanying interim consolidated financial statements other than the following:

On June 6, 2019, the Company effected a 9.95-for-one reverse split of the Company's issued and outstanding common and convertible preferred stock (see Note 7). All common share, convertible preferred share and per share information and related information included in the accompanying interim consolidated financial statements have been adjusted retroactively, where applicable, to reflect the reverse split.

On June 6, 2019, the Company's stockholders approved the 2019 Equity Incentive Award Plan ("2019 Plan") which will become effective in connection with the effectiveness of the Company's proposed initial public offering. The 2019 Plan provides for the granting of equity-based awards. Upon the effectiveness of the Company's proposed initial public offering, 2,200,000 shares of common stock will become available for future issuance plus any reserved shares not issued or subject to outstanding grants under the 2014 Plan on the effective date of the 2019 Plan, for issuance pursuant to awards granted under the 2019 Plan. The provisions of the 2019 Plan allow for periodic automatic increases for shares reserved under the 2019 Plan.

On June 6, 2019, the Company's stockholders approved the 2019 Employee Stock Purchase Plan ("2019 ESPP") which will become effective upon the effectiveness of the Company's proposed initial public offering. The 2019 ESPP initially provides for the issuance of up to 315,000 shares of common stock to employees. The provisions of the 2019 ESPP provide for automatic periodic increases for shares reserved under the 2019 ESPP.

On June 6, 2019, the Company's stockholders approved an amendment to the Company's Amended and Restated Certificate of Incorporation which will become effective in connection with the effectiveness of the Company's proposed initial public offering, to provide that the authorized capital stock of the Corporation shall consist of 300 million shares of common stock, $0.0001 per share par value, and 10 million shares of undesignated preferred stock, $0.0001 per share par value.

Table of Contents

6,700,000 shares

Common stock

Prospectus

J.P. Morgan

Cowen

Credit Suisse

Canaccord Genuity

, 2019

PART II

Information not required in prospectus

Item 13. Other expenses of issuance and distribution.

The following table sets forth all costs and expenses, other than underwriting discounts and commissions, paid or payable by the Registrant in connection with the sale of the common stock being registered. All amounts shown are estimates except for the SEC registration fee, the FINRA filing fee and the Nasdaq listing fee:

	Amount paid or to be paid
SEC registration fee	$ 14,942
FINRA filing fee	18,992
Nasdaq listing fee	125,000
Printing and engraving expenses	237,150
Legal fees, Blue Sky fees and expenses	1,500,000
Accounting fees and expenses	500,000
Transfer agent and registrar fees and expenses	6,500
Miscellaneous expenses	47,416
Total	$ 2,450,000

* To be completed by amendment.

Item 14. Indemnification of directors and officers.

Section 145 of the Delaware General Corporation Law, or DGCL, authorizes a court to award, or a corporation's board of directors to grant, indemnity to directors and officers under certain circumstances and subject to certain limitations. The terms of Section 145 of the DGCL are sufficiently broad to permit indemnification under certain circumstances for liabilities, including reimbursement of expenses incurred, arising under the Securities Act of 1933, as amended, or the Securities Act.

As permitted by the DGCL, the Registrant's restated certificate of incorporation to be effective in connection with the completion of this offering contains provisions that eliminate the personal liability of its directors for monetary damages for any breach of fiduciary duties as a director, except liability for the following:

- any breach of the director's duty of loyalty to the Registrant or its stockholders;
- acts or omissions not in good faith or that involve intentional misconduct or a knowing violation of law;
- under Section 174 of the DGCL (regarding unlawful dividends and stock purchases); or
- any transaction from which the director derived an improper personal benefit.

As permitted by the DGCL, the Registrant's restated bylaws to be effective in connection with the completion of this offering, provide that:

- the Registrant is required to indemnify its directors and executive officers to the fullest extent permitted by the DGCL, subject to limited exceptions;
- the Registrant may indemnify its other employees and agents as set forth in the DGCL;

- the Registrant is required to advance expenses, as incurred, to its directors and executive officers in connection with a legal proceeding to the fullest extent permitted by the DGCL, subject to limited exceptions; and
- the rights conferred in the restated bylaws are not exclusive.

Prior to the completion of this offering, the Registrant intends to enter into indemnification agreements with each of its current directors and executive officers to provide these directors and executive officers additional contractual assurances regarding the scope of the indemnification set forth in the Registrant's restated certificate of incorporation and restated bylaws and to provide additional procedural protections. There is no pending litigation or proceeding involving a director or executive officer of the Registrant for which indemnification is sought. Reference is also made to the underwriting agreement to be filed as Exhibit 1.1 to this registration statement, which provides for the indemnification of executive officers, directors and controlling persons of the Registrant against certain liabilities. The indemnification provisions in the Registrant's restated certificate of incorporation, restated bylaws and the indemnification agreements entered into or to be entered into between the Registrant and each of its directors and executive officers may be sufficiently broad to permit indemnification of the Registrant's directors and executive officers for liabilities arising under the Securities Act.

The Registrant has directors' and officers' liability insurance for securities matters.

Item 15. Recent sales of unregistered securities.

The following lists set forth information regarding all securities sold or granted by the Registrant from June 5, 2016 through June 5, 2019 that were not registered under the Securities Act, and the consideration, if any, received by the Registrant for such securities:

(a) Stock Option Grants

From June 5, 2016 through June 5, 2019, the Registrant has granted to its employees, directors, consultants and other service providers options to purchase an aggregate of 4,020,772 shares of common stock under its 2014 Equity Incentive Plan, or 2014 Plan, with exercise prices ranging from $0.40 to $15.00 per share.

From June 5, 2016 through June 5, 2019, employees, directors, consultants and other service providers of the Registrant exercised options granted under the 2014 Plan for an aggregate of 244,726 shares of common stock with exercise prices ranging from $0.40 to $2.19 per share for an aggregate exercise price of $118,184.

(b) Preferred Stock

In July 2016, the Registrant issued and sold an aggregate of 1,260,802 additional shares of Series A convertible preferred stock, at a purchase price of $2.38 per share, for an aggregate purchase price of approximately $3 million. In February 2017, we sold an aggregate of 1,260,802 additional shares of our Series A convertible preferred stock, at a purchase price of $2.38 per share, for an aggregate purchase price of approximately $3 million. Upon the completion of this offering, these shares of Series A convertible preferred stock will convert into 2,521,604 shares of common stock.

Between January and September 2018, the Registrant issued and sold to an investor an aggregate of 7,617,746 shares of Series A-2 convertible preferred stock, at a purchase price of $3.81 per share, for an aggregate purchase price of $29,000,000. Upon the completion of this offering, these shares of Series A-2 convertible preferred stock will convert into 7,617,746 shares of common stock.

On October 22, 2018, the Registrant issued and sold to 14 investors an aggregate of 10,079,671 shares of Series B convertible preferred stock, at a purchase price of $8.93 per share, for an aggregate purchase price of $89,999,994. Upon the completion of this offering, these shares of Series B convertible preferred stock will convert into 10,079,671 shares of common stock.

(c) Simple Agreement for Future Equity

In October 2017, we issued rights to purchase certain shares of our capital stock at a purchase price of $3,000,000, or Purchase Amount, pursuant to a Simple Agreement for Future Equity, or SAFE. The entire Purchase Amount and all other obligations under the SAFE converted into 788,042 shares of our Series A-2 convertible preferred stock in January 2018.

Unless otherwise stated, the sales of the above securities were deemed to be exempt from registration under the Securities Act in reliance on Section 4(a)(2) of the Securities Act (or Regulation D or Regulation S promulgated thereunder), or Rule 701 promulgated under Section 3(b) of the Securities Act, as transactions by an issuer not involving any public offering or pursuant to benefit plans and contracts relating to compensation as provided under Rule 701. The recipients of the securities in each of these transactions represented their intentions to acquire the securities for investment only and not with a view to or for sale in connection with any distribution thereof, and appropriate legends were placed on the stock certificates issued in each of the foregoing transactions.

None of the foregoing transactions involved any underwriters, underwriting discounts or commissions or any public offering, and the Registrant believes each transaction was exempt from the registration requirements of the Securities Act as stated above. All recipients of the foregoing transactions either received adequate information about the Registrant or had access, through their relationships with the Registrant, to such information. Furthermore, the Registrant affixed appropriate legends to the share certificates and instruments issued in each foregoing transaction setting forth that the securities had not been registered and the applicable restrictions on transfer.

Item 16. Exhibits and financial statement schedules.

(a) Exhibits.

Exhibit Number	Description of document
1.1	Form of Underwriting Agreement.
3.1	Amended and Restated Certificate of Incorporation, as amended to date, as currently in effect.
3.2*	Form of Restated Certificate of Incorporation to be effective upon the completion of this offering.
3.3*	Amended and Restated Bylaws, as amended to date, as currently in effect.
3.4*	Form of Restated Bylaws to be effective upon the completion of this offering.
4.1	Form of Common Stock Certificate.
4.2*	Amended and Restated Investors' Rights Agreement, dated October 22, 2018, by and among the Registrant and certain of its stockholders.
5.1	Opinion of Fenwick & West LLP.
10.1	Form of Indemnification Agreement with directors and officers.
10.2*	2014 Equity Incentive Plan, as amended, and forms of award agreements.
10.4	2019 Equity Incentive Plan, to become effective on the date immediately prior to the date the registration statement is declared effective, and forms of award agreements.
10.5	2019 Employee Stock Purchase Plan, to become effective on the date the registration statement is declared effective, and forms of award agreements.
10.6*	Lease Agreement, dated August 20, 2018, by and between Homology Medicines, Inc., and the Registrant.
10.7*	Lease Agreement dated January 2, 2019, by and between MIT 139 Main Street Leasehold LLC., and the Registrant.
10.8†*	License Agreement, dated July 31, 2015, by and between Cold Spring Harbor Laboratory and the Registrant.
10.9†*	License Agreement, dated April 18, 2016, by and between the University of Southampton and the Registrant.
10.10*	Employment Agreement, dated October 5, 2017, by and between the Registrant and Edward M. Kaye, as amended.
10.11*	Employment Agreement, dated November 20, 2015, by and between the Registrant and Huw M. Nash.
10.12*	Employment Agreement, dated September 8, 2017, by and between the Registrant and Barry J. Ticho, as amended.
10.13*	Employment Agreement, dated January 7, 2018, by and between the Registrant and Gene Liau.
10.14*	Employment Agreement, dated February 12, 2019, by and between the Registrant and Stephen J. Tulipano.
10.15*	Consulting Agreement, dated October 24, 2014, by and between the Registrant and Adrian R. Krainer.
21.1*	Subsidiaries of the Registrant.
23.1	Consent of KPMG, an independent registered public accounting firm.
23.2	Consent of Fenwick & West LLP (included in Exhibit 5.1).
23.3*	Consent of Health Advances LLC.
24.1*	Power of Attorney (included in the signature page to this registration statement).

* Previously filed.
† Confidential treatment requested with respect to portions of this exhibit.

(b) Financial Statement Schedules.

No financial statement schedules are provided because the information called for is not required or is shown either in the financial statements or notes.

Item 17. Undertakings.

The undersigned Registrant hereby undertakes to provide to the underwriters at the completion specified in the underwriting agreement, certificates in such denominations and registered in such names as required by the underwriters to permit prompt delivery to each purchaser.

Insofar as indemnification for liabilities arising under the Securities Act may be permitted to directors, officers and controlling persons of the Registrant pursuant to the foregoing provisions, or otherwise, the Registrant has been advised that in the opinion of the SEC such indemnification is against public policy as expressed in the Securities Act and is, therefore, unenforceable. In the event that a claim for indemnification against such liabilities (other than the payment by the Registrant of expenses incurred or paid by a director, officer or controlling person of the Registrant in the successful defense of any action, suit or proceeding) is asserted by such director, officer or controlling person in connection with the securities being registered, the Registrant will, unless in the opinion of its counsel the matter has been settled by controlling precedent, submit to a court of appropriate jurisdiction the question whether such indemnification by it is against public policy as expressed in the Securities Act and will be governed by the final adjudication of such issue.

The undersigned Registrant hereby undertakes that:

(1) For purposes of determining any liability under the Securities Act, the information omitted from the form of prospectus filed as part of this registration statement in reliance upon Rule 430A and contained in a form of prospectus filed by the Registrant pursuant to Rule 424(b)(1) or (4) or 497(h) under the Securities Act shall be deemed to be part of this registration statement as of the time it was declared effective.

(2) For the purpose of determining any liability under the Securities Act, each post-effective amendment that contains a form of prospectus shall be deemed to be a new registration statement relating to the securities offered therein, and the offering of such securities at that time shall be deemed to be the initial bona fide offering thereof.

Signatures

Pursuant to the requirements of the Securities Act of 1933, as amended, the Registrant has duly caused this registration statement on Form S-1 to be signed on its behalf by the undersigned, thereunto duly authorized, in the City of Bedford, State of Massachusetts, on the 7th day of June, 2019.

STOKE THERAPEUTICS, INC.

By: /s/ Edward M. Kaye
Edward M. Kaye, M.D.
Chief Executive Officer

Power of attorney

KNOW ALL PERSONS BY THESE PRESENTS, that each person whose signature appears below hereby constitutes and appoints Edward M. Kaye and Stephen J. Tulipano, and each of them, as his true and lawful attorneys-in-fact, proxies and agents, each with full power of substitution and resubstitution and full power to act without the other, for him in any and all capacities, to sign any and all amendments to this registration statement (including post-effective amendments or any abbreviated registration statement and any amendments thereto filed pursuant to Rule 462(b) increasing the number of securities for which registration is sought), and to file the same, with all exhibits thereto and other documents in connection therewith, with the Securities and Exchange Commission, granting unto said attorneys-in-fact, proxies and agents full power and authority to do and perform each and every act and thing requisite and necessary to be done in connection therewith, as fully for all intents and purposes as he or she might or could do in person, hereby ratifying and confirming all that said attorneys-in-fact, proxies and agents, or their or his or her substitute or substitutes, may lawfully do or cause to be done by virtue hereof.

Pursuant to the requirements of the Securities Act of 1933, as amended, this registration statement on Form S-1 has been signed by the following persons in the capacities and on the dates indicated.

Signature	Title	Date
/s/ Edward M. Kaye Edward M. Kaye, M.D.	Chief Executive Officer (Principal Executive Officer)	June 7, 2019
/s/ Stephen J. Tulipano Stephen J. Tulipano	Chief Financial Officer (Principal Accounting and Financial Officer)	June 7, 2019
/s/ Jennifer C. Burstein Jennifer C. Burstein	Director	June 7, 2019
* Samuel W. Hall, Ph.D.	Director	June 7, 2019
* Seth L. Harrison, M.D.	Director	June 7, 2019
* Adrian R. Krainer, Ph.D.	Director	June 7, 2019
* Arthur A. Levin, Ph.D.	Director	June 7, 2019

*	Director	June 7, 2019
Arthur O. Tzianabos, Ph.D.		

*By Attorney-in-Fact
/s/ Stephen J. Tulipano
Stephen J. Tulipano

EXHIBIT 1.1

Stoke Therapeutics, Inc.

[—] Shares of Common Stock

Underwriting Agreement

June [—], 2019

J.P. Morgan Securities LLC
Cowen and Company, LLC
Credit Suisse Securities (USA) LLC

As Representatives of the
several Underwriters listed
in Schedule 1 hereto

c/o J.P. Morgan Securities LLC
383 Madison Avenue
New York, New York 10179

c/o Cowen and Company, LLC
599 Lexington Avenue
New York, New York 10022

c/o Credit Suisse Securities (USA) LLC
Eleven Madison Avenue
New York, New York 10010-3629

Ladies and Gentlemen:

Stoke Therapeutics, Inc., a Delaware corporation (the "**Company**"), proposes to issue and sell to the several underwriters listed in Schedule 1 hereto (the "**Underwriters**"), for whom you are acting as representatives (the "**Representatives**"), an aggregate of [—] shares of common stock, par value $0.0001 per share, of the Company (the "**Underwritten Shares**") and, at the option of the Underwriters, up to an additional [—] shares of common stock of the Company (the "**Option Shares**"). The Underwritten Shares and the Option Shares are herein referred to as the "**Shares**". The shares of common stock of the Company to be outstanding after giving effect to the sale of the Shares are referred to herein as the "**Stock**".

The Company hereby confirms its agreement with the several Underwriters concerning the purchase and sale of the Shares, as follows:

1. Registration Statement. The Company has prepared and filed with the Securities and Exchange Commission (the "**Commission**") under the Securities Act of 1933, as amended, and the rules and regulations of the Commission thereunder (collectively, the "**Securities Act**"), a registration statement (File No. 333-231700), including a prospectus, relating to the Shares. Such registration statement, as amended at the time it became effective, including the information, if any, deemed pursuant to Rule 430A, 430B or 430C under the Securities Act to be part of the registration statement at the time of its effectiveness ("**Rule 430 Information**"), is referred to herein as the "**Registration Statement**"; and as used herein, the term "**Preliminary Prospectus**" means each prospectus included in such registration statement (and any amendments thereto) before effectiveness, any prospectus filed with the Commission

pursuant to Rule 424(a) under the Securities Act and the prospectus included in the Registration Statement at the time of its effectiveness that omits Rule 430 Information, and the term "**Prospectus**" means the prospectus in the form first used (or made available upon request of purchasers pursuant to Rule 173 under the Securities Act) in connection with confirmation of sales of the Shares. If the Company has filed an abbreviated registration statement pursuant to Rule 462(b) under the Securities Act (the "**Rule 462 Registration Statement**"), then any reference herein to the term "**Registration Statement**" shall be deemed to include such Rule 462 Registration Statement. Capitalized terms used but not defined herein shall have the meanings given to such terms in the Registration Statement and the Prospectus.

At or prior to the Applicable Time (as defined below), the Company had prepared the following information (collectively with the pricing information set forth in Part I of Annex A, the "**Pricing Disclosure Package**"): a Preliminary Prospectus dated June [—], 2019 and any "free-writing prospectus" (as defined pursuant to Rule 405 under the Securities Act) listed in Part II of Annex A hereto.

"**Applicable Time**" means [ ] P.M., New York City time, on June [—], 2019.

2. Purchase of the Shares.

(a) The Company agrees to issue and sell the Underwritten Shares to the several Underwriters as provided in this underwriting agreement (this "**Agreement**"), and each Underwriter, on the basis of the representations, warranties and agreements set forth herein and subject to the conditions set forth herein, agrees, severally and not jointly, to purchase at a price per share of $[—] (the "**Purchase Price**") from the Company the respective number of Underwritten Shares set forth opposite such Underwriter's name in Schedule 1 hereto.

In addition, the Company agrees to issue and sell the Option Shares to the several Underwriters as provided in this Agreement, and the Underwriters, on the basis of the representations, warranties and agreements set forth herein and subject to the conditions set forth herein, shall have the option to purchase, severally and not jointly, from the Company the Option Shares at the Purchase Price less an amount per share equal to any dividends or distributions declared by the Company and payable on the Underwritten Shares but not payable on the Option Shares.

If any Option Shares are to be purchased, the number of Option Shares to be purchased by each Underwriter shall be the number of Option Shares which bears the same ratio to the aggregate number of Option Shares being purchased as the number of Underwritten Shares set forth opposite the name of such Underwriter in Schedule 1 hereto (or such number increased as set forth in Section 10 hereof) bears to the aggregate number of Underwritten Shares being purchased from the Company by the several Underwriters, subject, however, to such adjustments to eliminate any fractional Shares as the Representatives in their sole discretion shall make.

The Underwriters may exercise the option to purchase Option Shares at any time in whole, or from time to time in part, on or before the thirtieth day following the date of the Prospectus, by written notice from the Representatives to the Company. Such notice shall set forth the aggregate number of Option Shares as to which the option is being exercised and the date and time when the Option Shares are to be delivered and paid for, which may be the same date and time as the Closing Date (as hereinafter defined) but shall not be earlier than the Closing Date nor later than the tenth full business day (as hereinafter defined) after the date of such notice (unless such time and date are postponed in accordance with the provisions of Section 10 hereof). Any such notice shall be given at least two business days prior to the date and time of delivery specified therein.

(b) The Company understands that the Underwriters intend to make a public offering of the Shares and, initially, to offer the Shares on the terms set forth in the Pricing Disclosure Package. The Company acknowledges and agrees that the Underwriters may offer and sell Shares to or through any affiliate of an Underwriter.

(c) Payment for the Shares shall be made by wire transfer in immediately available funds to the account specified by the Company to the Representatives in the case of the Underwritten Shares through the offices of Davis Polk & Wardwell LLP, 450 Lexington Avenue, New York, New York 10017 at 10:00 A.M. New York City time on June [—], 2019, or at such other time or place on the same or such other date, not later than the fifth business day thereafter, as the Representatives and the Company may agree upon in writing or, in the case of the Option Shares, on the date and at the time and place specified by the Representatives in the written notice of the Underwriters' election to purchase such Option Shares. The time and date of such payment for the Underwritten Shares is referred to herein as the "**Closing Date**", and the time and date for such payment for the Option Shares, if other than the Closing Date, is herein referred to as the "**Additional Closing Date**".

Payment for the Shares to be purchased on the Closing Date or the Additional Closing Date, as the case may be, shall be made against delivery to the Representatives for the respective accounts of the several Underwriters of the Shares to be purchased on such date or the Additional Closing Date, as the case may be, with any transfer taxes payable in connection with the sale of such Shares duly paid by the Company. Delivery of the Shares shall be made through the facilities of The Depository Trust Company ("**DTC**") unless the Representatives shall otherwise instruct.

(d) The Company acknowledges and agrees that the Representatives and the other Underwriters are acting solely in the capacity of an arm's length contractual counterparty to the Company with respect to the offering of Shares contemplated hereby (including in connection with determining the terms of the offering) and not as a financial advisor or a fiduciary to, or an agent of, the Company or any other person. Additionally, neither the Representatives nor any other Underwriter is advising the Company or any other person as to any legal, tax, investment, accounting or regulatory matters in any jurisdiction. The Company shall consult with its own advisors concerning such matters and shall be responsible for making its own independent investigation and appraisal of the transactions contemplated hereby, and neither the Representatives nor the other Underwriters shall have any responsibility or liability to the Company with respect thereto. Any review by the Representatives and the other Underwriters of the Company, the transactions contemplated hereby or other matters relating to such transactions will be performed solely for the benefit of the Underwriters and shall not be on behalf of the Company.

3. Representations and Warranties of the Company. The Company represents and warrants to each Underwriter that:

(a) *Preliminary Prospectus.* No order preventing or suspending the use of any Preliminary Prospectus has been issued by the Commission, and each Preliminary Prospectus included in the Pricing Disclosure Package, at the time of filing thereof, complied in all material respects with the Securities Act, and no Preliminary Prospectus, at the time of filing thereof, contained any untrue statement of a material fact or omitted to state a material fact necessary in order to make the statements therein, in the light of the circumstances under which they were made, not misleading; provided that the Company makes no representation or warranty with respect to any statements or omissions made in reliance upon and in conformity with information relating to any Underwriter furnished to the Company in writing by such Underwriter through the Representatives expressly for use in any Preliminary Prospectus, it being understood and agreed that the only such information furnished by any Underwriter consists of the information described as such in Section 7(b) hereof.

(b) *Pricing Disclosure Package*. The Pricing Disclosure Package as of the Applicable Time did not, and as of the Closing Date and as of the Additional Closing Date, as the case may be, will not, contain any untrue statement of a material fact or omit to state a material fact necessary in order to make the statements therein, in the light of the circumstances under which they were made, not misleading; provided that the Company makes no representation or warranty with respect to any statements or omissions made in reliance upon and in conformity with information relating to any Underwriter furnished to the Company in writing by such Underwriter through the Representatives expressly for use in such Pricing Disclosure Package, it being understood and agreed that the only such information furnished by any Underwriter consists of the information described as such in Section 7(b) hereof. No statement of material fact included in the Prospectus has been omitted from the Pricing Disclosure Package and no statement of material fact included in the Pricing Disclosure Package that is required to be included in the Prospectus has been omitted therefrom.

(c) *Issuer Free Writing Prospectus.* Other than the Registration Statement, the Preliminary Prospectus and the Prospectus, the Company (including its agents and representatives, other than the Underwriters in their capacity as such) has not prepared, made, used, authorized, approved or referred to and will not prepare, make, use, authorize, approve or refer to any "written communication" (as defined in Rule 405 under the Securities Act) that constitutes an offer to sell or solicitation of an offer to buy the Shares (each such communication by the Company or its agents and representatives (other than a communication referred to in clause (i) below) an "**Issuer Free Writing Prospectus**") other than (i) any document not constituting a prospectus pursuant to Section 2(a)(10)(a) of the Securities Act or Rule 134 under the Securities Act or (ii) the documents listed in Part II of Annex A hereto, each electronic road show and any other written communications approved in writing in advance by the Representatives, such approval not to be unreasonably withheld or delayed. Each such Issuer Free Writing Prospectus, if any, complies in all material respects with the Securities Act, has been or will be (within the time period specified in Rule 433 under the Securities Act) filed in accordance with the Securities Act (to the extent required thereby) and does not conflict with the information contained in the Registration Statement or the Pricing Disclosure Package, and, when taken together with any other Issuer Free Writing Prospectus and the Preliminary Prospectus, in each case, accompanying, or delivered prior to delivery of such Issuer Free Writing Prospectus, did not, and as of the Closing Date and as of the Additional Closing Date, as the case may be, will not, contain any untrue statement of a material fact or omit to state a material fact necessary in order to make the statements therein, in the light of the circumstances under which they were made, not misleading; provided that the Company makes no representation or warranty with respect to any statements or omissions made in each such Issuer Free Writing Prospectus or Preliminary Prospectus in reliance upon and in conformity with information relating to any Underwriter furnished to the Company in writing by such Underwriter through the Representatives expressly for use in such Issuer Free Writing Prospectus or Preliminary Prospectus, it being understood and agreed that the only such information furnished by any Underwriter consists of the information described as such in Section 7(b) hereof.

(d) *Emerging Growth Company*. From the time of initial confidential submission of the Registration Statement to the Commission (or, if earlier, the first date on which the Company engaged directly or through any person authorized to act on its behalf in any Testing-the-Waters Communication) through the date hereof, the Company has been and is an "emerging growth company," as defined in Section 2(a) of the Securities Act (an "**Emerging Growth Company**"). "**Testing-the-Waters Communication**" means any oral or written communication with potential investors undertaken in reliance on Section 5(d) of the Securities Act.

(e) *Testing-the-Waters Materials.* The Company (i) has not alone engaged in any Testing-the-Waters Communications, other than Testing-the-Waters Communications with the consent of the Representatives with entities that are qualified institutional buyers within the meaning of Rule 144A under the Securities Act or institutions that are accredited investors within the meaning of Rule 501 under the Securities Act, and (ii) has not authorized anyone other than the Representatives to engage in Testing-the-Waters Communications. The Company reconfirms that the Representatives have been authorized to act on its behalf in undertaking Testing-the-Waters Communications by virtue of a writing substantially in the form of Exhibit A hereto. The Company has not distributed or approved for distribution any Written Testing-the-Waters Communications other than those listed on Annex B hereto. "Written Testing-the-Waters Communication" means any Testing-the-Waters Communication that is a written communication within the meaning of Rule 405 under the Securities Act. Any individual Written Testing-the-Waters Communication does not conflict with the information contained in the Registration Statement or the Pricing Disclosure Package, complied in all material respects with the Securities Act, and when taken together with the Pricing Disclosure Package as of the Applicable Time, did not, and as of the Closing Date and as of the Additional Closing Date, as the case may be, will not, contain any untrue statement of a material fact or omit to state a material fact necessary in order to make the statements therein, in the light of the circumstances under which they were made, not misleading.

(f) *Registration Statement and Prospectus.* The Registration Statement has been declared effective by the Commission. No order suspending the effectiveness of the Registration Statement has been issued by the Commission, and no proceeding for that purpose or pursuant to Section 8A of the Securities Act against the Company or related to the offering of the Shares has been initiated or, to the knowledge of the Company, threatened by the Commission; as of the applicable effective date of the Registration Statement and any post-effective amendment thereto, the Registration Statement and any such post-effective amendment complied and will comply in all material respects with the Securities Act, and did not and will not contain any untrue statement of a material fact or omit to state a material fact required to be stated therein or necessary in order to make the statements therein not misleading; and as of the date of the Prospectus and any amendment or supplement thereto and as of the Closing Date and as of the Additional Closing Date, as the case may be, the Prospectus will comply in all material respects with the Securities Act and will not contain any untrue statement of a material fact or omit to state a material fact necessary in order to make the statements therein, in the light of the circumstances under which they were made, not misleading; provided that the Company makes no representation or warranty with respect to any statements or omissions made in reliance upon and in conformity with information relating to any Underwriter furnished to the Company in writing by such Underwriter through the Representatives expressly for use in the Registration Statement and the Prospectus and any amendment or supplement thereto, it being understood and agreed that the only such information furnished by any Underwriter consists of the information described as such in Section 7(b) hereof.

(g) *Financial Statements.* The financial statements (including the related notes thereto) of the Company and its consolidated subsidiaries included in the Registration Statement, the Pricing Disclosure Package and the Prospectus comply in all material respects with the applicable requirements of the Securities Act and present fairly, in all material respects, the financial position of the Company and its consolidated subsidiaries as of the dates indicated and the results of their operations and the changes in their cash flows for the periods specified; such

financial statements have been prepared in conformity with generally accepted accounting principles ("**GAAP**") in the United States applied on a consistent basis throughout the periods covered thereby, and any supporting schedules included in the Registration Statement present fairly, in all material respects, the information required to be stated therein; and the other financial information included in the Registration Statement, the Pricing Disclosure Package and the Prospectus has been derived from the accounting records of the Company and its consolidated subsidiaries and presents fairly, in all material respects, the information shown thereby. If applicable, the disclosure included in the Registration Statement, the Pricing Disclosure Package and the Prospectus regarding "non-GAAP financial measures" (as such term is defined by the rules and regulations of the Commission) complies, to the extent applicable, with Regulation G of the Exchange Act and Item 10 of Regulation S-K of the Securities Act.

(h) *No Material Adverse Change.* Since the date of the most recent financial statements of the Company included in the Registration Statement, the Pricing Disclosure Package and the Prospectus, (i) there has not been any change in the capital stock (other than the issuance of shares of common stock upon exercise of stock options and warrants described as outstanding in, and the grant of options and awards under existing equity incentive plans described in, the Registration Statement, the Pricing Disclosure Package and the Prospectus), short-term debt or long-term debt of the Company or any of its subsidiaries, or any dividend or distribution of any kind declared, set aside for payment, paid or made by the Company on any class of capital stock, or any material adverse change, or any development that would reasonably be expected to result in a material adverse change, in or affecting the business, properties, management, financial position, stockholders' equity, results of operations or prospects of the Company and its subsidiaries taken as a whole; (ii) neither the Company nor any of its subsidiaries has entered into any transaction or agreement (whether or not in the ordinary course of business) that is material to the Company and its subsidiaries taken as a whole or incurred any liability or obligation, direct or contingent, that is material to the Company and its subsidiaries taken as a whole; and (iii) neither the Company nor any of its subsidiaries has sustained any loss or interference with its business that is material to the Company and its subsidiaries taken as a whole and that is either from fire, explosion, flood or other calamity, whether or not covered by insurance, or from any labor disturbance or dispute or any action, order or decree of any court or arbitrator or governmental or regulatory authority, except in each case as otherwise disclosed in the Registration Statement, the Pricing Disclosure Package and the Prospectus.

(i) *Organization and Good Standing.* The Company and each of its subsidiaries have been duly organized and are validly existing and in good standing under the laws of their respective jurisdictions of organization, are duly qualified to do business and are in good standing in each jurisdiction in which their respective ownership or lease of property or the conduct of their respective businesses requires such qualification, and have all power and authority necessary to own or hold their respective properties and to conduct the businesses in which they are engaged, except where the failure to be so qualified or in good standing or have such power or authority would not, individually or in the aggregate, reasonably be expected to have a material adverse effect on the business, properties, management, financial position, stockholders' equity, results of operations or prospects of the Company and its subsidiaries taken as a whole or on the performance by the Company of its obligations under this Agreement (a "**Material Adverse Effect**"). The Company does not own or control, directly or indirectly, any corporation, association or other entity other than the subsidiaries listed in Exhibit 21 to the Registration Statement.

(j) *Capitalization.* The Company has an authorized capitalization as set forth in the Registration Statement, the Pricing Disclosure Package and the Prospectus under the heading "Capitalization"; all the outstanding shares of capital stock of the Company have been duly and validly authorized and issued and are fully paid and non-assessable and are not subject to any pre-emptive or similar rights; except as described in or expressly contemplated by the Registration Statement, the Pricing Disclosure Package and the Prospectus, there are no outstanding rights (including, without limitation, pre-emptive rights), warrants or options to acquire, or instruments convertible into or exchangeable for, any shares of capital stock or other equity interest in the Company or any of its subsidiaries, or any contract, commitment, agreement, understanding or arrangement of any kind relating to the issuance of any capital stock of the Company or any such subsidiary, any such convertible or exchangeable securities or any such rights, warrants or options. The capital stock of the Company conforms in all material respects to the description thereof contained in the Registration Statement, the Pricing Disclosure Package and the Prospectus; and all the outstanding shares of capital stock or other equity interests of each subsidiary owned, directly or indirectly, by the Company have been duly and validly authorized and issued, are fully paid and non-assessable and are owned directly or indirectly by the Company, free and clear of any lien, charge, encumbrance, security interest, restriction on voting or transfer or any other claim of any third party.

(k) *Stock Options.* With respect to the stock options (the "**Stock Options**") granted pursuant to the stock-based compensation plans of the Company and its subsidiaries (the "**Company Stock Plans**"), (i) each Stock Option intended to qualify as an "incentive stock option" under Section 422 of the Internal Revenue Code of 1986, as amended (the "**Code**") so qualifies, (ii) each grant of a Stock Option was duly authorized no later than the date on which the grant of such Stock Option was by its terms to be effective by all necessary corporate action, including, as applicable, approval by the board of directors of the Company (or a duly constituted and authorized committee thereof) and any required stockholder approval by the necessary number of votes or written consents, and the award agreement governing such grant (if any) was duly executed and delivered by each party thereto, (iii) each such grant was made in accordance with the Company Stock Plans, the applicable terms of the Exchange Act and all other applicable laws and regulatory rules or requirements, including the rules of any exchange on which Company securities may be traded, and (iv) each such grant was properly accounted for in accordance with GAAP in the financial statements (including the related notes) of the Company. The Company has not knowingly granted, and there is no and has been no policy or practice of the Company of granting, Stock Options prior to, or otherwise coordinating the grant of Stock Options with, the release or other public announcement of material information regarding the Company or its subsidiaries or their results of operations or prospects.

(l) *Due Authorization.* The Company has full right, power and authority to execute and deliver this Agreement and to perform its obligations hereunder; and all action required to be taken for the due and proper authorization, execution and delivery by it of this Agreement and the consummation by it of the transactions contemplated hereby has been duly and validly taken.

(m) *Underwriting Agreement.* This Agreement has been duly authorized, executed and delivered by the Company.

(n) *The Shares.* The Shares to be issued and sold by the Company hereunder have been duly authorized by the Company and, when issued and delivered and paid for as provided herein, will be duly and validly issued, will be fully paid and nonassessable and will conform to the descriptions thereof in the Registration Statement, the Pricing Disclosure Package and the Prospectus; and the issuance of the Shares is not subject to any preemptive or similar rights that have not been duly waived or satisfied.

(o) *Description of the Underwriting Agreement.* This Agreement conforms in all material respects to the description thereof contained in the Registration Statement, the Pricing Disclosure Package and the Prospectus.

(p) *No Violation or Default.* Neither the Company nor any of its subsidiaries is (i) in violation of its charter or by-laws or similar organizational documents; (ii) in default, and no event has occurred that, with notice or lapse of time or both, would constitute such a default, in the due performance or observance of any term, covenant or condition contained in any indenture, mortgage, deed of trust, loan agreement or other agreement or instrument to which the Company or any of its subsidiaries is a party or by which the Company or any of its subsidiaries is bound or to which any property or asset of the Company or any of its subsidiaries is subject; or (iii) in violation of any law or statute or any judgment, order, rule or regulation of any court or arbitrator or governmental or regulatory authority, except, in the case of clauses (ii) and (iii) above, for any such default or violation that would not, individually or in the aggregate, reasonably be expected to have a Material Adverse Effect.

(q) *No Conflicts.* The execution, delivery and performance by the Company of this Agreement, the issuance and sale of the Shares and the consummation of the transactions contemplated by this Agreement or the Pricing Disclosure Package and the Prospectus will not (i) conflict with or result in a breach or violation of any of the terms or provisions of, or constitute a default under, result in the termination, modification or acceleration of, or result in the creation or imposition of any lien, charge or encumbrance upon any property, right or asset of the Company or any of its subsidiaries pursuant to, any indenture, mortgage, deed of trust, loan agreement or other agreement or instrument to which the Company or any of its subsidiaries is a party or by which the Company or any of its subsidiaries is bound or to which any property, right or asset of the Company or any of its subsidiaries is subject, (ii) result in any violation of the provisions of the charter or by-laws or similar organizational documents of the Company or any of its subsidiaries or (iii) result in the violation of any law or statute or any judgment, order, rule or regulation of any court or arbitrator or governmental or regulatory authority, except, in the case of clauses (i) and (iii) above, for any such conflict, breach, violation, default, lien, charge or encumbrance that would not, individually or in the aggregate, have a Material Adverse Effect.

(r) *No Consents Required.* No consent, approval, authorization, order, registration or qualification of or with any court or arbitrator or governmental or regulatory authority is required for the execution, delivery and performance by the Company of this Agreement, the issuance and sale of the Shares and the consummation of the transactions contemplated by this Agreement, except for (i) the registration of the Shares under the Securities Act, (ii) such consents, approvals, authorizations, orders and registrations or qualifications as may be required by the Financial Industry Regulatory Authority, Inc. ("**FINRA**") and under applicable state securities laws in connection with the purchase and distribution of the Shares by the Underwriters and (iii) the filing of a restated certificate of incorporation of the Company with the Secretary of State of the State of Delaware.

(s) *Legal Proceedings.* Except as described in the Registration Statement, the Pricing Disclosure Package and the Prospectus, there are no legal, governmental or regulatory investigations, actions, demands, claims, suits, arbitrations, inquiries or proceedings ("**Actions**") pending to which the Company or any of its subsidiaries is a party or to which any property of the Company or any of its subsidiaries is the subject that, individually or in the aggregate, if determined adversely to the Company or any of its subsidiaries, would reasonably be expected to have a Material Adverse Effect; no such Actions are, to the knowledge of the Company, threatened or contemplated by any governmental or regulatory authority or threatened by others;

and (i) there are no current or pending Actions that are required under the Securities Act to be described in the Registration Statement, the Pricing Disclosure Package or the Prospectus that are not so described in the Registration Statement, the Pricing Disclosure Package and the Prospectus and (ii) there are no statutes, regulations or contracts or other documents that are required under the Securities Act to be filed as exhibits to the Registration Statement or described in the Registration Statement, the Pricing Disclosure Package or the Prospectus that are not so filed as exhibits to the Registration Statement or described in the Registration Statement, the Pricing Disclosure Package and the Prospectus.

(t) *Independent Accountants*. KPMG LLP, who have certified certain financial statements of the Company and its subsidiaries, is an independent registered public accounting firm with respect to the Company and its subsidiaries within the applicable rules and regulations adopted by the Commission and the Public Company Accounting Oversight Board (United States) and as required by the Securities Act.

(u) *Title to Real and Personal Property*. The Company and its subsidiaries have good and marketable title in fee simple to, or have valid rights to lease or otherwise use, all items of real and personal property that are material to the respective businesses of the Company and its subsidiaries, in each case free and clear of all liens, encumbrances, claims and defects and imperfections of title except those that (i) do not materially interfere with the use made and proposed to be made of such property by the Company and its subsidiaries or (ii) would not reasonably be expected, individually or in the aggregate, to have a Material Adverse Effect.

(v) *Intellectual Property.* Except as described in the Registration Statement, the Pricing Disclosure Package and the Prospectus and as would not, individually or in the aggregate, have a Material Adverse Effect: (i) the Company and its subsidiaries own, have adequate rights to use, or can acquire on reasonable terms all patents, trademarks, service marks, trade names, domain names and other source indicators, copyrights and copyrightable works, know-how, trade secrets, systems, procedures, proprietary or confidential information and all other worldwide intellectual property (including all registrations and applications for registration of, and all goodwill associated with, any of the foregoing) (collectively, "**Intellectual Property**") used in or necessary for the conduct of their respective businesses as now conducted or as contemplated in the Registration Statement, Pricing Disclosure Package and Prospectus to be conducted by them; (ii) the Company is unaware of any facts which would form a reasonable basis for an action, suit, proceeding or claim asserting that the Company has infringed, misappropriated or otherwise violated, or would upon the commercialization of any product described in the Registration Statement, the Pricing Disclosure Package or the Prospectus as under development infringe, misappropriate or otherwise violate, any Intellectual Property of any person or entity; (iii) to the knowledge of the Company, all Intellectual Property owned by or exclusively licensed to the Company and its subsidiaries is valid and enforceable; (iv) to the knowledge of the Company, the Intellectual Property of the Company and its subsidiaries is not being infringed, misappropriated or otherwise violated, and has not been infringed, misappropriated or otherwise violated, by any person or entity; (v) the Company and its subsidiaries have taken reasonable steps in accordance with normal industry practice to maintain the confidentiality of all Intellectual Property the value of which to the Company or any of its subsidiaries is contingent upon maintaining the confidentiality thereof, and to the knowledge of the Company, no such Intellectual Property has been disclosed other than to employees, representatives and agents of the Company or any of its subsidiaries, all of whom are bound by written confidentiality agreements, (vi) there is no pending or, to the knowledge of the Company, threatened action, suit, proceeding or claim by any third party (A) challenging the Company's or any of its subsidiaries' rights in or to any Intellectual Property, (B) challenging the validity, enforceability or scope of any Intellectual Property owned

by the Company or any of its subsidiaries, or (C) alleging that the Company or any of its subsidiaries has infringed, misappropriated or otherwise violated any Intellectual Property of any third party, (vii) to the knowledge of the Company, there is no pending or threatened action, suit proceeding or claim by any third party challenging the validity, enforceability or scope of any Intellectual Property exclusively licensed to the Company or any of its subsidiaries and (viii) each agreement pursuant to which the Company or any of its subsidiaries obtains any license or other rights to any Intellectual Property is a valid and binding agreement of the Company and its subsidiaries and is in full force and effect, and none of the Company or any of its subsidiaries or, to the knowledge of the Company, any other party to any such agreement, is in default or breach under any terms of any such agreement and, to the knowledge of the Company, no event or circumstance has occurred that, with notice or lapse of time or both, would constitute any event of default thereunder.

(w) *Preclinical Studies.* (i) Except as described in the Registration Statement, the Pricing Disclosure Package and the Prospectus, the preclinical studies conducted by or, to the knowledge of the Company, on behalf of or sponsored by the Company or its subsidiaries, or in which the Company or its subsidiaries have participated, that are described in the Registration Statement, the Pricing Disclosure Package and the Prospectus, or the results of which are referred to in the Registration Statement, the Pricing Disclosure Package and the Prospectus, as applicable, were, and if still pending, are, being conducted in all material respects in accordance with standard medical and scientific research standards and procedures for products or product candidates comparable to those being developed by the Company and all applicable statutes, rules and regulations of the U.S. Food and Drug Administration and comparable regulatory agencies outside of the United States to which they are subject, including the European Medicines Agency (collectively, the "**Regulatory Authorities**") and all applicable statutes, rules and regulations of the Regulatory Authorities and Good Clinical Practice and Good Laboratory Practice requirements; (ii) the descriptions in the Registration Statement, the Pricing Disclosure Package and the Prospectus of the results of such studies are accurate and complete descriptions in all material respects and fairly present the data derived therefrom; (iii) the Company has no knowledge of any other studies not described in the Registration Statement, the Pricing Disclosure Package and the Prospectus, the results of which are inconsistent with or call into question the results described or referred to in the Registration Statement, the Pricing Disclosure Package and the Prospectus; (iv) the Company and its subsidiaries have operated at all times and are currently in compliance in all respects with all applicable statutes, rules and regulations of the Regulatory Authorities, except that where such non-compliance would not, individually or in the aggregate, have a Material Adverse Effect; (v) the Company has provided the Underwriters with all substantive written notices, correspondence and summaries of all other communications from the Regulatory Authorities; and (vi) neither the Company nor any of its subsidiaries have received any written notices, correspondence or other communications from the Regulatory Authorities or any other governmental agency requiring or threatening the termination, material modification or suspension of any preclinical studies that are described in the Registration Statement, the Pricing Disclosure Package and the Prospectus or the results of which are referred to in the Registration Statement, the Pricing Disclosure Package and the Prospectus, other than ordinary course communications with respect to modifications in connection with the design and implementation of such studies or planned clinical trials, and, to the Company's knowledge, there are no reasonable grounds for the same.

(x) *Regulatory Filings*. The Company has not failed to file with the Regulatory Authorities any required filing, declaration, listing, registration, report or submission with respect to the Company's product candidates that are described or referred to in the Registration Statement, the Pricing Disclosure Package and the Prospectus. All such filings, declarations, listings, registrations, reports or submissions, as applicable, were in material compliance with applicable laws when filed; and no material deficiencies regarding compliance with applicable law have been asserted by any applicable Regulatory Authority with respect to any such filings, declarations, listings, registrations, reports or submissions.

(y) *No Undisclosed Relationships*. No relationship, direct or indirect, exists between or among the Company or any of its subsidiaries, on the one hand, and the directors, officers, stockholders, customers, suppliers or other affiliates of the Company or any of its subsidiaries, on the other, that is required by the Securities Act to be described in each of the Registration Statement and the Prospectus and that is not so described in such documents and in the Pricing Disclosure Package.

(z) *Investment Company Act*. The Company is not and, after giving effect to the offering and sale of the Shares and the application of the proceeds thereof as described in the Registration Statement, the Pricing Disclosure Package and the Prospectus, will not be required to register as an "investment company" or an entity "controlled" by an "investment company" within the meaning of the Investment Company Act of 1940, as amended, and the rules and regulations of the Commission thereunder (collectively, the "**Investment Company Act**").

(aa) *Taxes.* The Company and its subsidiaries have paid all income and other material federal, state, local and foreign taxes and filed all tax returns required to be paid or filed through the date hereof; and except as otherwise disclosed in each of the Registration Statement, the Pricing Disclosure Package and the Prospectus, there is no tax deficiency that has been, or would reasonably be expected to be, asserted against the Company or any of its subsidiaries or any of their respective properties or assets and which would reasonably be expected to have, individually or in the aggregate, a Material Adverse Effect.

(bb) *Licenses and Permits.* The Company and its subsidiaries possess all licenses, sub-licenses, certificates, permits and other authorizations issued by, and have made all declarations and filings with, the appropriate federal, state, local or foreign governmental or regulatory authorities that are necessary for the ownership or lease of their respective properties or the conduct of their respective businesses as described in each of the Registration Statement, the Pricing Disclosure Package and the Prospectus, except where the failure to possess or make the same would not, individually or in the aggregate, reasonably be expected to have a Material Adverse Effect; and except as described in each of the Registration Statement, the Pricing Disclosure Package and the Prospectus, neither the Company nor any of its subsidiaries has received notice of any revocation or modification of any such license, sub-license, certificate, permit or authorization or has any reason to believe that any such license, sub-license, certificate, permit or authorization will not be renewed in the ordinary course.

(cc) *No Labor Disputes.* No labor disturbance by or dispute with employees of the Company or any of its subsidiaries exists or, to the knowledge of the Company, is contemplated or threatened, and the Company is not aware of any existing or imminent labor disturbance by, or dispute with, the employees of any of its or its subsidiaries' principal suppliers, contractors or customers, except as would not have a Material Adverse Effect. Neither the Company nor any of its subsidiaries has received any notice of cancellation or termination with respect to any collective bargaining agreement to which it is a party.

(dd) *Certain Environmental Matters*. (i) The Company and its subsidiaries (x) are in compliance with all, and have not violated any, applicable federal, state, local and foreign laws (including common law), rules, regulations, requirements, decisions, judgments, decrees, orders and other legally enforceable requirements relating to pollution or the protection of human health or safety, the environment, natural resources, hazardous or toxic substances or wastes, pollutants

or contaminants (collectively, "**Environmental Laws**"); (y) have received and are in compliance with all, and have not violated any, permits, licenses, certificates or other authorizations or approvals required of them under any Environmental Laws to conduct their respective businesses; and (z) have not received notice of any actual or potential liability or obligation under or relating to, or any actual or potential violation of, any Environmental Laws, including for the investigation or remediation of any disposal or release of hazardous or toxic substances or wastes, pollutants or contaminants, and have no knowledge of any event or condition that would reasonably be expected to result in any such notice, and (ii) there are no costs or liabilities associated with Environmental Laws of or relating to the Company or its subsidiaries, except in the case of each of (i) and (ii) above, for any such matter as would not, individually or in the aggregate, reasonably be expected to have a Material Adverse Effect; and (iii) except as described in each of the Pricing Disclosure Package and the Prospectus, (x) there is no proceeding that is pending, or that is known to be contemplated, against the Company or any of its subsidiaries under any Environmental Laws in which a governmental entity is also a party, other than such proceeding regarding which it is reasonably believed no monetary sanctions of $100,000 or more will be imposed, (y) the Company and its subsidiaries are not aware of any facts or issues regarding compliance with Environmental Laws, or liabilities or other obligations under Environmental Laws or concerning hazardous or toxic substances or wastes, pollutants or contaminants, that would reasonably be expected to have a material effect on the capital expenditures, earnings or competitive position of the Company and its subsidiaries, and (z) none of the Company or its subsidiaries anticipates material capital expenditures relating to any Environmental Laws.

(ee) *Compliance with ERISA*. (i) Each employee benefit plan, within the meaning of Section 3(3) of the Employee Retirement Income Security Act of 1974, as amended ("**ERISA**"), for which the Company or any member of its "Controlled Group" (defined as any entity, whether or not incorporated, that is under common control with the Company within the meaning of Section 4001(a)(14) of ERISA or any entity that would be regarded as a single employer with the Company under Section 414(b),(c),(m) or (o) of the Code would have any liability (each, a "**Plan**") has been maintained in compliance with its terms and the requirements of any applicable statutes, orders, rules and regulations, including but not limited to ERISA and the Code; (ii) no prohibited transaction, within the meaning of Section 406 of ERISA or Section 4975 of the Code, has occurred with respect to any Plan, excluding transactions effected pursuant to a statutory or administrative exemption; (iii) for each Plan that is subject to the funding rules of Section 412 of the Code or Section 302 of ERISA, no Plan has failed (whether or not waived), or is reasonably expected to fail, to satisfy the minimum funding standards (within the meaning of Section 302 of ERISA or Section 412 of the Code) applicable to such Plan; (iv) no Plan is, or is reasonably expected to be, in "at risk status" (within the meaning of Section 303(i) of ERISA) and no Plan that is a "multiemployer plan" within the meaning of Section 4001(a)(3) of ERISA is in "endangered status" or "critical status" (within the meaning of Sections 304 and 305 of ERISA) (v) the fair market value of the assets of each Plan exceeds the present value of all benefits accrued under such Plan (determined based on those assumptions used to fund such Plan); (vi) no "reportable event" (within the meaning of Section 4043(c) of ERISA and the regulations promulgated thereunder) has occurred or is reasonably expected to occur; (vii) each Plan that is intended to be qualified under Section 401(a) of the Code is so qualified, and nothing has occurred, whether by action or by failure to act, which would cause the loss of such qualification; (viii) neither the Company nor any member of the Controlled Group has incurred, nor reasonably expects to incur, any liability under Title IV of ERISA (other than contributions to the Plan or premiums to the Pension Benefit Guarantee Corporation, in the ordinary course and without default) in respect of a Plan (including a "multiemployer plan" within the meaning of Section 4001(a)(3) of ERISA); and (ix) none of the following events has occurred or is reasonably likely

to occur: (A) a material increase in the aggregate amount of contributions required to be made to all Plans by the Company or its Controlled Group affiliates in the current fiscal year of the Company and its Controlled Group affiliates compared to the amount of such contributions made in the Company's and its Controlled Group affiliates' most recently completed fiscal year; or (B) a material increase in the Company and its subsidiaries' "accumulated post-retirement benefit obligations" (within the meaning of Accounting Standards Codification Topic 715-60) compared to the amount of such obligations in the Company and its subsidiaries' most recently completed fiscal year, except in each case with respect to the events or conditions set forth in (i) through (ix) hereof, as would not, individually or in the aggregate, have a Material Adverse Effect.

(ff) *Disclosure Controls*. The Company (on a consolidated basis with its subsidiary) maintains an effective system of "disclosure controls and procedures" (as defined in Rule 13a-15(e) of the Exchange Act) that complies with the applicable requirements of the Exchange Act and that has been designed to ensure that information required to be disclosed by the Company in reports that it files or submits under the Exchange Act is recorded, processed, summarized and reported within the time periods specified in the Commission's rules and forms, including controls and procedures designed to ensure that such information is accumulated and communicated to the Company's management as appropriate to allow timely decisions regarding required disclosure.

(gg) *Accounting Controls.* The Company (on a consolidated basis with its subsidiary) maintains systems of "internal control over financial reporting" (as defined in Rule 13a-15(f) of the Exchange Act) that comply with the applicable requirements of the Exchange Act and have been designed by, or under the supervision of, their respective principal executive and principal financial officers, or persons performing similar functions, to provide reasonable assurance regarding the reliability of financial reporting and the preparation of financial statements for external purposes in accordance with GAAP. The Company and its subsidiaries maintain internal accounting controls sufficient to provide reasonable assurance that (i) transactions are executed in accordance with management's general or specific authorizations; (ii) transactions are recorded as necessary to permit preparation of financial statements in conformity with GAAP and to maintain asset accountability; (iii) access to assets is permitted only in accordance with management's general or specific authorization; and (iv) the recorded accountability for assets is compared with the existing assets at reasonable intervals and appropriate action is taken with respect to any differences. There are no material weaknesses in the Company's internal controls. The Company's auditors and the Audit Committee of the Board of Directors of the Company have been advised of: (i) all significant deficiencies and material weaknesses, if any, in the design or operation of internal controls over financial reporting which have adversely affected or are reasonably likely to adversely affect the Company's ability to record, process, summarize and report financial information; and (ii) any fraud, whether or not material, that involves management or other employees who have a significant role in the Company's internal controls over financial reporting.

(hh) *Insurance*. The Company and its subsidiaries have insurance covering their respective properties, operations, personnel and businesses, including business interruption insurance, which insurance is in amounts and insures against such losses and risks as are generally maintained by companies engaged in the same or similar businesses and at the same or a similar stage of development, and which the Company reasonably believes are adequate to protect the Company and its business; and neither the Company nor any of its subsidiaries has (i) received notice from any insurer or agent of such insurer that capital improvements or other expenditures are required or necessary to be made in order to continue such insurance or (ii) any reason to believe that it will not be able to renew its existing insurance coverage as and when such coverage expires or to obtain similar coverage at reasonable cost from similar insurers as may be necessary to continue its business.

(ii) *Cybersecurity; Data Protection.* The Company and its subsidiaries' information technology assets and equipment, computers, systems, networks, hardware, software, websites, applications, and databases (collectively, "**IT Systems**") are adequate for, and operate and perform in all material respects as required in connection with, the operation of the business of the Company and its subsidiaries as currently conducted, free and clear of all material bugs, errors, defects, Trojan horses, time bombs, malware and other corruptants. The Company and its subsidiaries have implemented and maintained commercially reasonable controls, procedures and safeguards to maintain and protect their material confidential information and Personal Data (defined below) and the integrity and security of all material IT Systems. As used herein, "**Personal Data**" shall refer to all personal or regulated data that relates to an identified or identifiable natural person according to applicable law that is maintained by the Company or any of its subsidiaries and used in connection with the Company or its subsidiaries' respective businesses. To the knowledge of the Company, there have been no material breaches, violations, outages or unauthorized uses of or access to Personal Data, except for those that have been remedied without material cost or liability, nor any related incidents under investigation by a governmental or regulatory authority. To the knowledge of the Company, the Company and its subsidiaries are presently in material compliance with all applicable laws, internal policies and contractual obligations relating to the privacy and security of IT Systems and Personal Data and to the protection of IT Systems and Personal Data from unauthorized use, access, misappropriation or modification.

(jj) *No Unlawful Payments.* Neither the Company nor any of its subsidiaries, nor, to the knowledge of the Company, any director, officer, employee of the Company or any of its subsidiaries, or any agent, affiliate or other person associated with or acting on behalf of the Company or any of its subsidiaries has (i) used any corporate funds for any unlawful contribution, gift, entertainment or other unlawful expense relating to political activity; (ii) made or taken an act in furtherance of an offer, promise or authorization of any direct or indirect unlawful payment or benefit to any foreign or domestic government official or employee, including of any government-owned or controlled entity or of a public international organization, or any person acting in an official capacity for or on behalf of any of the foregoing, or any political party or party official or candidate for political office; (iii) violated or is in violation of any provision of the Foreign Corrupt Practices Act of 1977, as amended, or any applicable law or regulation implementing the OECD Convention on Combating Bribery of Foreign Public Officials in International Business Transactions, or committed an offence under the Bribery Act 2010 of the United Kingdom or any other applicable anti-bribery or anti-corruption law; or (iv) made, offered, agreed, requested or taken an act in furtherance of any unlawful bribe or other unlawful benefit, including, without limitation, any rebate, payoff, influence payment, kickback or other unlawful or improper payment or benefit. The Company and its subsidiaries have instituted, maintain and enforce, and will continue to maintain and enforce policies and procedures designed to promote and ensure compliance with all applicable anti-bribery and anti-corruption laws.

(kk) *Compliance with Anti-Money Laundering Laws*. The operations of the Company and its subsidiaries are and have been conducted at all times in compliance with applicable financial recordkeeping and reporting requirements, including those of the Currency and Foreign Transactions Reporting Act of 1970, as amended, the applicable money laundering statutes of all jurisdictions where the Company or any of its subsidiaries conducts business, the rules and regulations thereunder and any related or similar rules, regulations or guidelines issued, administered or enforced by any governmental agency (collectively, the "**Anti-Money**

Laundering Laws") and no action, suit or proceeding by or before any court or governmental agency, authority or body or any arbitrator involving the Company or any of its subsidiaries with respect to the Anti-Money Laundering Laws is pending or, to the knowledge of the Company, threatened.

(ll) *No Conflicts with Sanctions Laws.* Neither the Company nor any of its subsidiaries, nor, to the knowledge of the Company, any director, officer, employee, agent, affiliate or other person associated with or acting on behalf of the Company or any of its subsidiaries is currently the subject or the target of any sanctions administered or enforced by the U.S. government, (including, without limitation, the Office of Foreign Assets Control of the U.S. Department of the Treasury ("**OFAC**") or the U.S. Department of State and including, without limitation, the designation as a "specially designated national" or "blocked person"), the United Nations Security Council ("**UNSC**"), the European Union, Her Majesty's Treasury ("**HMT**") or other relevant sanctions authority (collectively, "**Sanctions**"), nor is the Company or any of its subsidiaries located, organized or resident in a country or territory that is the subject or target of Sanctions, including, without limitation, Crimea, Cuba, Iran, North Korea and Syria (each, a "**Sanctioned Country**"); and the Company will not directly or indirectly use the proceeds of the offering of the Shares hereunder, or lend, contribute or otherwise make available such proceeds to any subsidiary, joint venture partner or other person or entity (i) to fund or facilitate any activities of or business with any person that, at the time of such funding or facilitation, is the subject or target of Sanctions, (ii) to fund or facilitate any activities of or business in any Sanctioned Country or (iii) in any other manner that will result in a violation by any person (including any person participating in the transaction, whether as underwriter, advisor, investor or otherwise) of Sanctions. For the past five years, the Company and its subsidiaries have not knowingly engaged in and are not now knowingly engaged in any dealings or transactions with any person that at the time of the dealing or transaction is or was the subject or the target of Sanctions or with any Sanctioned Country.

(mm) *No Restrictions on Subsidiaries*. Subject to any restrictions under any applicable, laws, no subsidiary of the Company is currently prohibited, directly or indirectly, under any agreement or other instrument to which it is a party or is subject, from paying any dividends to the Company, from making any other distribution on such subsidiary's capital stock or similar ownership interest, from repaying to the Company any loans or advances to such subsidiary from the Company or from transferring any of such subsidiary's properties or assets to the Company or any other subsidiary of the Company.

(nn) *No Broker's Fees.* Neither the Company nor any of its subsidiaries is a party to any contract, agreement or understanding with any person (other than this Agreement) that would give rise to a valid claim against any of them or any Underwriter for a brokerage commission, finder's fee or like payment in connection with the offering and sale of the Shares.

(oo) *No Registration Rights.* Except as described in the Registration Statement, the Pricing Disclosure Package and the Prospectus, to the extent that any person has the right to require the Company or any of its subsidiaries to register any securities for sale under the Securities Act by reason of the filing of the Registration Statement with the Commission or the issuance and sale of the Shares, those rights have been waived as of the date of this Agreement with respect to such filing or issuance and sale of Shares pursuant to this Agreement.

(pp) *No Stabilization.* Neither the Company nor any of its subsidiaries or affiliates has taken, directly or indirectly, any action designed to or that would reasonably be expected to cause or result in any stabilization or manipulation of the price of the Shares.

(qq) *Margin Rules*. Neither the issuance, sale and delivery of the Shares nor the application of the proceeds thereof by the Company as described in each of the Registration Statement, the Pricing Disclosure Package and the Prospectus will violate Regulation T, U or X of the Board of Governors of the Federal Reserve System or any other regulation of such Board of Governors.

(rr) *Forward-Looking Statements.* No forward-looking statement (within the meaning of Section 27A of the Securities Act and Section 21E of the Exchange Act) included in any of the Registration Statement, the Pricing Disclosure Package or the Prospectus has been made or reaffirmed without a reasonable basis or has been disclosed other than in good faith.

(ss) *Statistical and Market Data.* Nothing has come to the attention of the Company that has caused the Company to believe that the statistical and market-related data included in each of the Registration Statement, the Pricing Disclosure Package and the Prospectus is not based on or derived from sources that are reliable and accurate in all material respects.

(tt) *Sarbanes-Oxley Act*. There is and has been no failure on the part of the Company or any of the Company's directors or officers, in their capacities as such, to comply with any provision of the Sarbanes-Oxley Act of 2002, as amended and the rules and regulations promulgated in connection therewith (the "**Sarbanes-Oxley Act**"), with which the Company is required to comply, including Section 402 related to loans.

(uu) *Status under the Securities Act.* At the time of filing the Registration Statement and any post-effective amendment thereto, at the earliest time thereafter that the Company or any offering participant made a *bona fide* offer (within the meaning of Rule 164(h)(2) under the Securities Act) of the Shares and at the date hereof, the Company was not and is not an "ineligible issuer," as defined in Rule 405 under the Securities Act. The Company has paid the registration fee for this offering pursuant to Rule 456(b)(1) under the Securities Act or will pay such fee within the time period required by such rule (without giving effect to the proviso therein) and in any event prior to the Closing Date.

(vv) *No Ratings*. There are (and prior to the Closing Date, will be) no debt securities, convertible securities or preferred stock issued or guaranteed by the Company or any of its subsidiaries that are rated by a "nationally recognized statistical rating organization", as such term is defined in Section 3(a)(62) under the Exchange Act.

4. Further Agreements of the Company. The Company covenants and agrees with each Underwriter that:

(a) *Required Filings.* The Company will file the final Prospectus with the Commission within the time periods specified by Rule 424(b) and Rule 430A, 430B or 430C under the Securities Act, will file any Issuer Free Writing Prospectus to the extent required by Rule 433 under the Securities Act, and the Company will furnish copies of the Prospectus and each Issuer Free Writing Prospectus (to the extent not previously delivered) to the Underwriters in New York City prior to 10:00 A.M., New York City time, on the business day next succeeding the date of this Agreement in such quantities as the Representatives may reasonably request.

(b) *Delivery of Copies.* The Company will deliver, upon request and without charge, (i) to the Representatives, three, signed copies of the Registration Statement as originally filed and each amendment thereto, in each case including all exhibits and consents filed therewith and (ii) to each Underwriter (A) a conformed copy of the Registration Statement as originally filed

and each amendment thereto (without exhibits) and (B) during the Prospectus Delivery Period (as defined below), as many copies of the Prospectus (including all amendments and supplements thereto and each Issuer Free Writing Prospectus) as the Representatives may reasonably request. As used herein, the term "Prospectus Delivery Period" means such period of time after the first date of the public offering of the Shares as in the opinion of counsel for the Underwriters a prospectus relating to the Shares is required by law to be delivered (or required to be delivered but for Rule 172 under the Securities Act) in connection with sales of the Shares by any Underwriter or dealer.

(c) *Amendments or Supplements, Issuer Free Writing Prospectuses.* Before making, preparing, using, authorizing, approving, referring to or filing any Issuer Free Writing Prospectus, and before filing any amendment or supplement to the Registration Statement, the Pricing Disclosure Package or the Prospectus, the Company will furnish to the Representatives and counsel for the Underwriters a copy of the proposed Issuer Free Writing Prospectus, amendment or supplement for review and will not make, prepare, use, authorize, approve, refer to or file any such Issuer Free Writing Prospectus or file any such proposed amendment or supplement to which the Representatives reasonably object in a timely manner.

(d) *Notice to the Representatives.* The Company will advise the Representatives promptly, and confirm such advice in writing (which may be by electronic mail), (i) when the Registration Statement has become effective; (ii) when any amendment to the Registration Statement has been filed or becomes effective; (iii) when any supplement to the Pricing Disclosure Package, the Prospectus, any Issuer Free Writing Prospectus or any Written Testing-the-Waters Communication or any amendment to the Prospectus has been filed or distributed; (iv) of any request by the Commission for any amendment to the Registration Statement or any amendment or supplement to the Prospectus or the receipt of any comments from the Commission relating to the Registration Statement or any other request by the Commission for any additional information including, but not limited to, any request for information concerning any Testing-the-Waters Communication; (v) of the issuance by the Commission or any other governmental or regulatory authority of any order suspending the effectiveness of the Registration Statement or preventing or suspending the use of any Preliminary Prospectus, any of the Pricing Disclosure Package, the Prospectus or any Written Testing-the-Waters Communication or the initiation or threatening of any proceeding for that purpose or pursuant to Section 8A of the Securities Act; (vi) of the occurrence of any event or development within the Prospectus Delivery Period as a result of which the Prospectus, any of the Pricing Disclosure Package, any Issuer Free Writing Prospectus or any Written Testing-the-Waters Communication as then amended or supplemented would include any untrue statement of a material fact or omit to state a material fact necessary in order to make the statements therein, in the light of the circumstances existing when the Prospectus, the Pricing Disclosure Package, any such Issuer Free Writing Prospectus or any Written Testing-the-Waters Communication is delivered to a purchaser, not misleading and (vii) of the receipt by the Company of any notice with respect to any suspension of the qualification of the Shares for offer and sale in any jurisdiction or the initiation or, to the Company's knowledge, threatening of any proceeding for such purpose; and the Company will use its reasonable best efforts to prevent the issuance of any such order suspending the effectiveness of the Registration Statement, preventing or suspending the use of any Preliminary Prospectus, any of the Pricing Disclosure Package or the Prospectus or any Written Testing-the-Waters Communication or suspending any such qualification of the Shares and, if any such order is issued, will obtain as soon as possible the withdrawal thereof.

(e) *Ongoing Compliance.* (1) If during the Prospectus Delivery Period (i) any event or development shall occur or condition shall exist as a result of which the Prospectus as then amended or supplemented would include any untrue statement of a material fact or omit to state any material fact necessary in order to make the statements therein, in the light of the circumstances existing when the Prospectus is delivered to a purchaser, not misleading or (ii) it is necessary to amend or supplement the Prospectus to comply with law, the Company will promptly notify the Underwriters thereof and forthwith prepare and, subject to paragraph (c) above, file with the Commission and furnish to the Underwriters and to such dealers as the Representatives may designate such amendments or supplements to the Prospectus as may be necessary so that the statements in the Prospectus as so amended or supplemented will not, in the light of the circumstances existing when the Prospectus is delivered to a purchaser, be misleading or so that the Prospectus will comply with law and (2) if at any time prior to the Closing Date (i) any event or development shall occur or condition shall exist as a result of which the Pricing Disclosure Package as then amended or supplemented would include any untrue statement of a material fact or omit to state any material fact necessary in order to make the statements therein, in the light of the circumstances existing when the Pricing Disclosure Package is delivered to a purchaser, not misleading or (ii) it is necessary to amend or supplement the Pricing Disclosure Package to comply with law, the Company will promptly notify the Underwriters thereof and forthwith prepare and, subject to paragraph (c) above, file with the Commission (to the extent required) and furnish to the Underwriters and to such dealers as the Representatives may designate such amendments or supplements to the Pricing Disclosure Package as may be necessary so that the statements in the Pricing Disclosure Package as so amended or supplemented will not, in the light of the circumstances existing when the Pricing Disclosure Package is delivered to a purchaser, be misleading or so that the Pricing Disclosure Package will comply with law.

(f) *Blue Sky Compliance.* The Company will qualify the Shares for offer and sale under the securities or Blue Sky laws of such jurisdictions as the Representatives shall reasonably request and will continue such qualifications in effect so long as required for distribution of the Shares; provided that the Company shall not be required to (i) qualify as a foreign corporation or other entity or as a dealer in securities in any such jurisdiction where it would not otherwise be required to so qualify, (ii) file any general consent to service of process in any such jurisdiction or (iii) subject itself to taxation in any such jurisdiction if it is not otherwise so subject.

(g) *Earning Statement.* The Company will make generally available to its security holders and the Representatives as soon as practicable an earning statement that satisfies the provisions of Section 11(a) of the Securities Act and Rule 158 of the Commission promulgated thereunder covering a period of at least twelve months beginning with the first fiscal quarter of the Company occurring after the "effective date" (as defined in Rule 158) of the Registration Statement; provided the Company will be deemed to have satisfied such requirement to the extent such information is filed on the Commission's Electronic Data Gathering, Analysis and Retrieval system ("**EDGAR**") or any successor thereto.

(h) *Clear Market.* For a period of 180 days after the date of the Prospectus, the Company will not (i) offer, pledge, announce the intention to sell, sell, contract to sell, sell any option or contract to purchase, purchase any option or contract to sell, grant any option, right or warrant to purchase or otherwise transfer or dispose of, directly or indirectly, or file with, or submit to, the Commission a registration statement under the Securities Act relating to, any shares of Stock or any securities convertible into or exchangeable or exercisable for any Stock, or publicly disclose the intention to make any offer, sale, pledge, disposition, submission or filing, or (ii) enter into any swap or other agreement that transfers all or a portion of the economic consequences associated with the ownership of any Stock or any such other securities, whether any such transaction described in clause (i) or (ii) above is to be settled by delivery of Stock or

such other securities, in cash or otherwise, without the prior written consent of J.P. Morgan Securities LLC, other than (a) the Shares to be sold hereunder, (b) any shares of Stock of the Company issued upon the exercise of options granted under Company Stock Plans, (c) the grant or issuance by the Company of employee, consultant, or director stock options or restricted stock in the ordinary course of business under the Company Stock Plans described in the Registration Statement, the Pricing Disclosure Package and the Prospectus, (d) shares registered on Form S-8 relating to the Company Stock Plans described in the Registration Statement, the Pricing Disclosure Package and the Prospectus, (e) the issuance of securities in connection with the acquisition by the Company or any of its subsidiaries of the securities, businesses, property or other assets of another person or entity or pursuant to any employee benefit plan assumed by the Company in connection with any such acquisition, or (f) the issuance of securities in connection with joint ventures, commercial relationships, or other strategic transactions; provided that, (x) in the case of clauses (e) and (f), the aggregate number of shares issued in all such acquisitions and transactions taken together does not exceed 5% of the Company's outstanding common stock following the offering of Common Stock contemplated by this Agreement and (y) each person to whom such shares or securities are issued or granted pursuant to clauses (b), (c), (d), (e) and (f) during the 180-day restriction period described above executes or has executed a "lock-up" agreement in the form of Exhibit D hereto. If J.P. Morgan Securities LLC , in its sole discretion, agrees to release or waive the restrictions set forth in a lock-up letter described in Section 6(n) hereof for an officer or director of the Company and provides the Company with notice of the impending release or waiver substantially in the form of Exhibit B hereto at least three business days before the effective date of the release or waiver, the Company agrees to announce the impending release or waiver by a press release substantially in the form of Exhibit C hereto through a major news service at least two business days before the effective date of the release or waiver.

(i) *Use of Proceeds.* The Company will apply the net proceeds from the sale of the Shares as described in each of the Registration Statement, the Pricing Disclosure Package and the Prospectus under the heading "Use of Proceeds".

(j) *No Stabilization.* Neither the Company nor its subsidiaries will take, directly or indirectly, any action designed to or that would reasonably be expected to cause or result in any stabilization or manipulation of the price of the Stock.

(k) *Exchange Listing.* The Company will use its reasonable best efforts to list, subject to notice of issuance, the Shares on the Nasdaq Global Market (the "**Exchange**").

(l) *Reports.* For a period of two years from the date of this Agreement, the Company will furnish to the Representatives, as soon as they are available, copies of all reports or other communications (financial or other) furnished to holders of the Shares, and copies of any reports and financial statements furnished to or filed with the Commission or any national securities exchange or automatic quotation system; provided the Company will be deemed to have furnished such reports and financial statements to the Representatives to the extent they are filed on EDGAR or any successor to such system.

(m) *Record Retention.* The Company will, pursuant to reasonable procedures developed in good faith, retain copies of each Issuer Free Writing Prospectus that is not filed with the Commission in accordance with Rule 433 under the Securities Act.

(n) *Filings.* The Company will file with the Commission such reports as may be required by Rule 463 under the Securities Act.

(o) *Emerging Growth Company*. The Company will promptly notify the Representatives if the Company ceases to be an Emerging Growth Company at any time prior to the later of (i) completion of the distribution of Shares within the meaning of the Securities Act and (ii) completion of the 180-day restricted period referred to in Section 4(h) hereof.

(p) *Tax Indemnity*. The Company will indemnify and hold harmless the Underwriters against any documentary, stamp, registration or similar issuance tax, including any interest and penalties, on the sale of the Shares by the Company to the Underwriters and on the execution and delivery of this Agreement.

5. Certain Agreements of the Underwriters. Each Underwriter hereby represents and agrees that:

(a) It has not and will not use, authorize use of, refer to or participate in the planning for use of, any "free writing prospectus", as defined in Rule 405 under the Securities Act (which term includes use of any written information furnished to the Commission by the Company and not incorporated by reference into the Registration Statement and any press release issued by the Company) other than (i) a free writing prospectus that contains no "issuer information" (as defined in Rule 433(h)(2) under the Securities Act) that was not included (including through incorporation by reference) in the Preliminary Prospectus or a previously filed Issuer Free Writing Prospectus, (ii) any Issuer Free Writing Prospectus listed in Part II of Annex A or prepared pursuant to Section 3(c) or Section 4(c) above (including any electronic road show), or (iii) any free writing prospectus prepared by such Underwriter and approved by the Company in advance in writing (each such free writing prospectus referred to in clauses (i) or (iii), an "**Underwriter Free Writing Prospectus**").

(b) It has not and will not, without the prior written consent of the Company, use any free writing prospectus that contains the final terms of the Shares unless such terms have previously been included in a free writing prospectus filed with the Commission; *provided* that Underwriters may use a term sheet substantially in the form of Annex C hereto without the consent of the Company; *provided further* that any Underwriter using such term sheet shall notify the Company, and provide a copy of such term sheet to the Company, prior to, or substantially concurrently with, the first use of such term sheet.

(c) It is not subject to any pending proceeding under Section 8A of the Securities Act with respect to the offering (and will promptly notify the Company if any such proceeding against it is initiated during the Prospectus Delivery Period).

6. Conditions of Underwriters' Obligations. The obligation of each Underwriter to purchase the Underwritten Shares on the Closing Date or the Option Shares on the Additional Closing Date, as the case may be, as provided herein is subject to the performance by the Company of its covenants and other obligations hereunder and to the following additional conditions:

(a) *Registration Compliance; No Stop Order.* No order suspending the effectiveness of the Registration Statement shall be in effect, and no proceeding for such purpose or pursuant to Section 8A under the Securities Act shall be pending before or threatened by the Commission; the Prospectus and each Issuer Free Writing Prospectus shall have been timely filed with the Commission under the Securities Act (in the case of an Issuer Free Writing Prospectus, to the extent required by Rule 433 under the Securities Act) and in accordance with Section 4(a) hereof; and all requests by the Commission for additional information shall have been complied with to the reasonable satisfaction of the Representatives.

(b) *Representations and Warranties.* The representations and warranties of the Company contained herein shall be true and correct on the date hereof and on and as of the Closing Date or the Additional Closing Date, as the case may be; and the statements of the Company and its officers made in any certificates delivered pursuant to this Agreement shall be true and correct on and as of the Closing Date or the Additional Closing Date, as the case may be.

(c) *No Material Adverse Change.* No event or condition of a type described in Section 3(h) hereof shall have occurred or shall exist, which event or condition is not described in the Pricing Disclosure Package (excluding any amendment or supplement thereto) and the Prospectus (excluding any amendment or supplement thereto) and the effect of which in the judgment of the Representatives makes it impracticable or inadvisable to proceed with the offering, sale or delivery of the Shares on the Closing Date or the Additional Closing Date, as the case may be, on the terms and in the manner contemplated by this Agreement, the Pricing Disclosure Package and the Prospectus.

(d) *Officer's Certificate.* The Representatives shall have received on and as of the Closing Date or the Additional Closing Date, as the case may be, a certificate of the chief financial officer or chief accounting officer of the Company and one additional senior executive officer of the Company who is satisfactory to the Representatives (i) confirming that such officers have carefully reviewed the Registration Statement, the Pricing Disclosure Package and the Prospectus and, to the knowledge of such officers, the representations set forth in Sections 3(b) and 3(d) hereof are true and correct, (ii) confirming that the other representations and warranties of the Company in this Agreement are true and correct and that the Company has complied in all material respects with all agreements and satisfied all conditions on its part to be performed or satisfied hereunder at or prior to the Closing Date or the Additional Closing Date, as the case may be, and (iii) to the effect set forth in paragraphs (a) and (c) above.

(e) *Chief Financial Officer's Certificate*. If requested by the Representatives, on the date of this Agreement and on the Closing Date or the Additional Closing Date, as the case may be, the Company shall have furnished to the Representatives a certificate, dated the respective dates of delivery thereof and addressed to the Representatives, of its chief financial officer with respect to certain financial data contained in the Pricing Disclosure Package and the Prospectus, providing "management comfort" with respect to such information, in form and substance reasonably satisfactory to the Representatives.

(f) *Comfort Letters.* On the date of this Agreement and on the Closing Date or the Additional Closing Date, as the case may be, KPMG LLP shall have furnished to the Representatives, at the request of the Company, letters, dated the respective dates of delivery thereof and addressed to the Underwriters, in form and substance reasonably satisfactory to the Representatives, containing statements and information of the type customarily included in accountants' "comfort letters" to underwriters with respect to the financial statements and certain financial information contained in each of the Registration Statement, the Pricing Disclosure Package and the Prospectus; provided, that the letter delivered on the Closing Date or the Additional Closing Date, as the case may be, shall use a "cut-off" date no more than three business days prior to such Closing Date or such Additional Closing Date, as the case may be.

(g) *Opinion and 10b-5 Statement of Counsel for the Company.* Fenwick & West, LLP, counsel for the Company, shall have furnished to the Representatives, at the request of the Company, their written opinion and 10b-5 statement, dated the Closing Date or the Additional Closing Date, as the case may be, and addressed to the Representatives, in form and substance reasonably satisfactory to the Representatives.

(h) *Intellectual Property Opinion of Counsel for the Company.* Wilson, Sonsini, Goodrich & Rosati, P.C., counsel for the Company with respect to intellectual property matters, shall have furnished to the Representatives, at the request of the Company, their written intellectual property opinion, dated the Closing Date or the Additional Closing Date, as the case may be, and addressed to the Representatives, in form and substance reasonably satisfactory to the Representatives.

(i) *Regulatory Opinion of Counsel for the Company.* Hyman, Phelps & McNamara, P.C., counsel for the Company with respect to regulatory matters, shall have furnished to the Representatives, at the request of the Company, their written regulatory opinion, dated the Closing Date or the Additional Closing Date, as the case may be, and addressed to the Representatives, in form and substance reasonably satisfactory to the Representatives.

(j) *Opinion and 10b-5 Statement of Counsel for the Underwriters.* The Representatives shall have received on and as of the Closing Date or the Additional Closing Date, as the case may be, an opinion and 10b-5 statement, addressed to the Representatives, of Davis Polk & Wardwell LLP, counsel for the Underwriters, with respect to such matters as the Representatives may reasonably request, and such counsel shall have received such documents and information as they may reasonably request to enable them to pass upon such matters.

(k) *No Legal Impediment to Issuance and Sale.* No action shall have been taken and no statute, rule, regulation or order shall have been enacted, adopted or issued by any federal, state or foreign governmental or regulatory authority that would, as of the Closing Date or the Additional Closing Date, as the case may be, prevent the issuance or sale of the Shares; and no injunction or order of any federal, state or foreign court shall have been issued that would, as of the Closing Date or the Additional Closing Date, as the case may be, prevent the issuance or sale of the Shares.

(l) *Good Standing*. The Representatives shall have received on and as of the Closing Date or the Additional Closing Date, as the case may be, satisfactory evidence of the good standing of the Company and its subsidiaries in their respective jurisdictions of organization and their good standing in such other jurisdictions as the Representatives may reasonably request, in each case in writing or any standard form of telecommunication from the appropriate governmental authorities of such jurisdictions.

(m) *Certificate Regarding Beneficial Ownership*. The Representatives shall have received, prior to the date of this Agreement, properly completed and executed Certifications Regarding Beneficial Ownership of Legal Entity Customers, together with copies of identifying documentation.

(n) *Exchange Listing.* The Shares to be delivered on the Closing Date or the Additional Closing Date, as the case may be, shall have been approved for listing on the Exchange, subject to official notice of issuance.

(o) *Lock-up Agreements*. The “lock-up” agreements, each substantially in the form of Exhibit D hereto, between the Representatives and shareholders, officers and directors of the Company relating to sales and certain other dispositions of shares of Stock or certain other securities, delivered to the Representatives on or before the date hereof, shall be in full force and effect on the Closing Date or the Additional Closing Date, as the case may be.

(p) *Additional Documents.* On or prior to the Closing Date or the Additional Closing Date, as the case may be, the Company shall have furnished to the Representatives such further certificates and documents as the Representatives may reasonably request.

All opinions, letters, certificates and evidence mentioned above or elsewhere in this Agreement shall be deemed to be in compliance with the provisions hereof only if they are in form and substance reasonably satisfactory to counsel for the Underwriters.

7. Indemnification and Contribution.

(a) *Indemnification of the Underwriters.* The Company agrees to indemnify and hold harmless each Underwriter, its affiliates, directors and officers and each person, if any, who controls such Underwriter within the meaning of Section 15 of the Securities Act or Section 20 of the Exchange Act, from and against any and all losses, claims, damages and liabilities (including, without limitation, reasonable and documented legal fees and other reasonable expenses incurred in connection with any suit, action or proceeding or any claim asserted, as such fees and expenses are incurred), joint or several, that arise out of, or are based upon, (i) any untrue statement or alleged untrue statement of a material fact contained in the Registration Statement or caused by any omission or alleged omission to state therein a material fact required to be stated therein or necessary in order to make the statements therein, not misleading, or (ii) any untrue statement or alleged untrue statement of a material fact contained in the Prospectus (or any amendment or supplement thereto), any Preliminary Prospectus, any Issuer Free Writing Prospectus, any "issuer information" filed or required to be filed pursuant to Rule 433(d) under the Securities Act, any Written Testing-the-Waters Communication, any road show as defined in Rule 433(h) under the Securities Act (a "**road show**") or any Pricing Disclosure Package (including any Pricing Disclosure Package that has subsequently been amended), or caused by any omission or alleged omission to state therein a material fact necessary in order to make the statements therein, in light of the circumstances under which they were made, not misleading, in each case except insofar as such losses, claims, damages or liabilities arise out of, or are based upon, any untrue statement or omission or alleged untrue statement or omission made in reliance upon and in conformity with any information relating to any Underwriter furnished to the Company in writing by such Underwriter through the Representatives expressly for use therein, it being understood and agreed that the only such information furnished by any Underwriter consists of the information described as such in subsection (b) below.

(b) *Indemnification of the Company.* Each Underwriter agrees, severally and not jointly, to indemnify and hold harmless the Company, its directors, its officers who signed the Registration Statement and each person, if any, who controls the Company within the meaning of Section 15 of the Securities Act or Section 20 of the Exchange Act to the same extent as the indemnity set forth in paragraph (a) above, but only with respect to any losses, claims, damages or liabilities that arise out of, or are based upon, any untrue statement or omission or alleged untrue statement or omission made in reliance upon and in conformity with any information relating to such Underwriter furnished to the Company in writing by such Underwriter through the Representatives expressly for use in the Registration Statement, the Prospectus (or any amendment or supplement thereto), any Preliminary Prospectus, any Issuer Free Writing Prospectus, any Written Testing-the-Waters Communication, any road show or any Pricing Disclosure Package (including any Pricing Disclosure Package that has subsequently been amended), it being understood and agreed upon that the only such information furnished by any Underwriter consists of the following information in the Prospectus under the heading "Underwriting" furnished on behalf of each Underwriter: the first, second and third sentences appearing in the third paragraph relating to concessions and reallowances and the information contained in the thirteenth, fourteenth and fifteenth paragraphs describing passive market making activities and stabilization.

(c) *Notice and Procedures.* If any suit, action, proceeding (including any governmental or regulatory investigation), claim or demand shall be brought or asserted against any person in respect of which indemnification may be sought pursuant to the preceding paragraphs of this Section 7, such person (the "**Indemnified Person**") shall promptly notify the person against whom such indemnification may be sought (the "**Indemnifying Person**") in writing; provided that the failure to notify the Indemnifying Person shall not relieve it from any liability that it may have under the preceding paragraphs of this Section 7 except to the extent that it has been materially prejudiced (through the forfeiture of substantive rights or defenses) by such failure; and provided, further, that the failure to notify the Indemnifying Person shall not relieve it from any liability that it may have to an Indemnified Person otherwise than under the preceding paragraphs of this Section 7. If any such proceeding shall be brought or asserted against an Indemnified Person and it shall have notified the Indemnifying Person thereof, the Indemnifying Person shall retain counsel reasonably satisfactory to the Indemnified Person (who shall not, without the consent of the Indemnified Person, be counsel to the Indemnifying Person) to represent the Indemnified Person and any others entitled to indemnification pursuant to this Section that the Indemnifying Person may designate in such proceeding and shall pay the reasonable and documented fees and expenses in such proceeding and shall pay the reasonable and documented fees and expenses of such counsel related to such proceeding, as incurred. In any such proceeding, any Indemnified Person shall have the right to retain its own counsel, but the fees and expenses of such counsel shall be at the expense of such Indemnified Person unless (i) the Indemnifying Person and the Indemnified Person shall have mutually agreed to the contrary; (ii) the Indemnifying Person has failed within a reasonable time to retain counsel reasonably satisfactory to the Indemnified Person; (iii) the Indemnified Person shall have reasonably concluded that there may be legal defenses available to it that are different from or in addition to those available to the Indemnifying Person; or (iv) the named parties in any such proceeding (including any impleaded parties) include both the Indemnifying Person and the Indemnified Person and representation of both parties by the same counsel would be inappropriate due to actual or potential differing interests between them. It is understood and agreed that the Indemnifying Person shall not, in connection with any proceeding or related proceeding in the same jurisdiction, be liable for the fees and expenses of more than one separate firm (in addition to any local counsel) for all Indemnified Persons, and that all such fees and expenses shall be paid or reimbursed as they are incurred. Any such separate firm for any Underwriter, its affiliates, directors and officers and any control persons of such Underwriter shall be designated in writing by J.P. Morgan Securities LLC, Cowen and Company, LLC and Credit Suisse Securities (USA) LLC and any such separate firm for the Company, its directors, its officers who signed the Registration Statement and any control persons of the Company shall be designated in writing by the Company. The Indemnifying Person shall not be liable for any settlement of any proceeding effected without its written consent, but if settled with such consent, the Indemnifying Person agrees to indemnify each Indemnified Person from and against any loss or liability by reason of such settlement. Notwithstanding the foregoing sentence, if at any time an Indemnified Person shall have requested that an Indemnifying Person reimburse the Indemnified Person for fees and expenses of counsel as contemplated by this paragraph, the Indemnifying Person shall be liable for any settlement of any proceeding effected without its written consent if (i) such settlement is entered into more than 30 days after receipt by the Indemnifying Person of such request and (ii) the Indemnifying Person shall not have reimbursed the Indemnified Person in accordance with such request prior to the date of such settlement. No Indemnifying Person shall, without the written consent of the Indemnified Person, effect any settlement of any pending or threatened proceeding in respect of which any Indemnified Person is or could have been a party and indemnification could have been sought hereunder by such Indemnified Person, unless such settlement (x) includes an unconditional release of such Indemnified Person, in form and substance reasonably satisfactory to such Indemnified Person, from all liability on claims that are the subject matter of such proceeding and (y) does not include any statement as to or any admission of fault, culpability or a failure to act by or on behalf of any Indemnified Person.

(d) *Contribution.* If the indemnification provided for in paragraphs (a) and (b) above is unavailable to an Indemnified Person or insufficient in respect of any losses, claims, damages or liabilities referred to therein, then each Indemnifying Person under such paragraph, in lieu of indemnifying such Indemnified Person thereunder, shall contribute to the amount paid or payable by such Indemnified Person as a result of such losses, claims, damages or liabilities (i) in such proportion as is appropriate to reflect the relative benefits received by the Company, on the one hand, and the Underwriters on the other, from the offering of the Shares or (ii) if the allocation provided by clause (i) is not permitted by applicable law, in such proportion as is appropriate to reflect not only the relative benefits referred to in clause (i) but also the relative fault of the Company, on the one hand, and the Underwriters on the other, in connection with the statements or omissions that resulted in such losses, claims, damages or liabilities, as well as any other relevant equitable considerations. The relative benefits received by the Company, on the one hand, and the Underwriters on the other, shall be deemed to be in the same respective proportions as the net proceeds (before deducting expenses) received by the Company from the sale of the Shares and the total underwriting discounts and commissions received by the Underwriters in connection therewith, in each case as set forth in the table on the cover of the Prospectus, bear to the aggregate offering price of the Shares. The relative fault of the Company, on the one hand, and the Underwriters on the other, shall be determined by reference to, among other things, whether the untrue or alleged untrue statement of a material fact or the omission or alleged omission to state a material fact relates to information supplied by the Company or by the Underwriters and the parties' relative intent, knowledge, access to information and opportunity to correct or prevent such statement or omission.

(e) *Limitation on Liability.* The Company and the Underwriters agree that it would not be just and equitable if contribution pursuant to paragraph (d) above were determined by pro rata allocation (even if the Underwriters were treated as one entity for such purpose) or by any other method of allocation that does not take account of the equitable considerations referred to in paragraph (d) above. The amount paid or payable by an Indemnified Person as a result of the losses, claims, damages and liabilities referred to in paragraph (d) above shall be deemed to include, subject to the limitations set forth above, any reasonable and documented legal or other expenses incurred by such Indemnified Person in connection with any such action or claim. Notwithstanding the provisions of paragraphs (d) and (e), in no event shall an Underwriter be required to contribute any amount in excess of the amount by which the total underwriting discounts and commissions received by such Underwriter with respect to the offering of the Shares exceeds the amount of any damages that such Underwriter has otherwise been required to pay by reason of such untrue or alleged untrue statement or omission or alleged omission. No person guilty of fraudulent misrepresentation (within the meaning of Section 11(f) of the Securities Act) shall be entitled to contribution from any person who was not guilty of such fraudulent misrepresentation. The Underwriters' obligations to contribute pursuant to paragraphs (d) and (e) are several in proportion to their respective purchase obligations hereunder and not joint.

(f) *Non-Exclusive Remedies.* The remedies provided for in this Section 7 paragraphs (a) through (e) are not exclusive and shall not limit any rights or remedies which may otherwise be available to any Indemnified Person at law or in equity.

8. Effectiveness of Agreement. This Agreement shall become effective as of the date first written above.

9. Termination. This Agreement may be terminated in the absolute discretion of the Representatives, by notice to the Company, if after the execution and delivery of this Agreement and on or prior to the Closing Date or, in the case of the Option Shares, prior to the Additional Closing Date (i) trading generally shall have been suspended or materially limited on or by any of the New York Stock Exchange or the Nasdaq Stock Market; (ii) trading of any securities issued or guaranteed by the Company shall have been suspended on any exchange or in any over-the-counter market; (iii) a general moratorium

on commercial banking activities shall have been declared by federal or New York State authorities; or (iv) there shall have occurred any outbreak or escalation of hostilities or any change in financial markets or any calamity or crisis, either within or outside the United States, that, in the judgment of the Representatives, is material and adverse and makes it impracticable or inadvisable to proceed with the offering, sale or delivery of the Shares on the Closing Date or the Additional Closing Date, as the case may be, on the terms and in the manner contemplated by this Agreement, the Pricing Disclosure Package and the Prospectus.

10. Defaulting Underwriter.

(a) If, on the Closing Date or the Additional Closing Date, as the case may be, any Underwriter defaults on its obligation to purchase the Shares that it has agreed to purchase hereunder on such date, the non-defaulting Underwriters may in their discretion arrange for the purchase of such Shares by other persons satisfactory to the Company on the terms contained in this Agreement. If, within 36 hours after any such default by any Underwriter, the non-defaulting Underwriters do not arrange for the purchase of such Shares, then the Company shall be entitled to a further period of 36 hours within which to procure other persons satisfactory to the non-defaulting Underwriters to purchase such Shares on such terms. If other persons become obligated or agree to purchase the Shares of a defaulting Underwriter, either the non-defaulting Underwriters or the Company may postpone the Closing Date or the Additional Closing Date, as the case may be, for up to five full business days in order to effect any changes that in the opinion of counsel for the Company or counsel for the Underwriters may be necessary in the Registration Statement and the Prospectus or in any other document or arrangement, and the Company agrees to promptly prepare any amendment or supplement to the Registration Statement and the Prospectus that effects any such changes. As used in this Agreement, the term "Underwriter" includes, for all purposes of this Agreement unless the context otherwise requires, any person not listed in Schedule 1 hereto that, pursuant to this Section 10, purchases Shares that a defaulting Underwriter agreed but failed to purchase.

(b) If, after giving effect to any arrangements for the purchase of the Shares of a defaulting Underwriter or Underwriters by the non-defaulting Underwriters and the Company as provided in paragraph (a) above, the aggregate number of Shares that remain unpurchased on the Closing Date or the Additional Closing Date, as the case may be, does not exceed one-eleventh of the aggregate number of Shares to be purchased on such date, then the Company shall have the right to require each non-defaulting Underwriter to purchase the number of Shares that such Underwriter agreed to purchase hereunder on such date plus such Underwriter's pro rata share (based on the number of Shares that such Underwriter agreed to purchase on such date) of the Shares of such defaulting Underwriter or Underwriters for which such arrangements have not been made.

(c) If, after giving effect to any arrangements for the purchase of the Shares of a defaulting Underwriter or Underwriters by the non-defaulting Underwriters and the Company as provided in paragraph (a) above, the aggregate number of Shares that remain unpurchased on the Closing Date or the Additional Closing Date, as the case may be, exceeds one-eleventh of the aggregate amount of Shares to be purchased on such date, or if the Company shall not exercise the right described in paragraph (b) above, then this Agreement or, with respect to any Additional Closing Date, the obligation of the Underwriters to purchase Shares on the Additional Closing Date, as the case may be, shall terminate without liability on the part of the non-defaulting Underwriters. Any termination of this Agreement pursuant to this Section 10 shall be without liability on the part of the Company, except that the Company will continue to be liable for the payment of expenses as set forth in Section 11 hereof and except that the provisions of Section 7 hereof shall not terminate and shall remain in effect.

(d) Nothing contained herein shall relieve a defaulting Underwriter of any liability it may have to the Company or any non-defaulting Underwriter for damages caused by its default.

11. Payment of Expenses.

(a) Whether or not the transactions contemplated by this Agreement are consummated or this Agreement is terminated, the Company will pay or cause to be paid all costs and expenses actually incurred and incident to the performance of its obligations hereunder, including without limitation, (i) the costs incident to the authorization, issuance, sale, preparation and delivery of the Shares and any taxes payable in that connection (other than, for the avoidance of doubt, taxes incident to the resale of the Shares by the Underwriters); (ii) the costs incident to the preparation, printing and filing under the Securities Act of the Registration Statement, the Preliminary Prospectus, any Issuer Free Writing Prospectus, any Pricing Disclosure Package and the Prospectus (including all exhibits, amendments and supplements thereto) and the distribution thereof; (iii) the fees and expenses of the Company's counsel and independent accountants; (iv) the fees and expenses incurred in connection with the registration or qualification and determination of eligibility for investment of the Shares under the laws of such jurisdictions as the Representatives may designate and the preparation, printing and distribution of a Blue Sky Memorandum (including the related fees and expenses of counsel for the Underwriters in an aggregate amount not to exceed $10,000); (v) the cost of preparing stock certificates; (vii) the costs and charges of any transfer agent and any registrar; (viii) all expenses and application fees incurred in connection with any filing with, and clearance of the offering by, FINRA (including the reasonable fees and expenses of counsel for the Underwriters related to such filings) in an aggregate amount not to exceed $35,000; (ix) all expenses incurred by the Company in connection with any road show presentation to potential investors, provided, however, that the Underwriters will pay all of the travel and lodging expenses of the Underwriters or any of their employees as incurred by them in connection with the road show, and provided, further that the Company and the Underwriters will each pay 50% of the cost of any aircraft chartered in connection with any road show and (x) all expenses and application fees related to the listing of the Shares on the Exchange.

(b) If (i) this Agreement is terminated pursuant to Section 9, (ii) the Company for any reason fails to tender the Shares for delivery to the Underwriters (other than by reason of a default by any Underwriter) or (iii) the Underwriters decline to purchase the Shares for any reason permitted under this Agreement, the Company agrees to reimburse the Underwriters for all documented and out-of-pocket costs and expenses (including the fees and expenses of their counsel) reasonably incurred by the Underwriters in connection with this Agreement and the offering contemplated hereby.

12. Persons Entitled to Benefit of Agreement. This Agreement shall inure to the benefit of and be binding upon the parties hereto and their respective successors and the officers and directors and any controlling persons referred to herein, and the affiliates of each Underwriter referred to in Section 7 hereof. Nothing in this Agreement is intended or shall be construed to give any other person any legal or equitable right, remedy or claim under or in respect of this Agreement or any provision contained herein. No purchaser of Shares from any Underwriter shall be deemed to be a successor merely by reason of such purchase.

13. Survival. The respective indemnities, rights of contribution, representations, warranties and agreements of the Company and the Underwriters contained in this Agreement or made by or on behalf of the Company or the Underwriters pursuant to this Agreement or any certificate delivered pursuant hereto shall survive the delivery of and payment for the Shares and shall remain in full force and effect, regardless of any termination of this Agreement or any investigation made by or on behalf of the Company or the Underwriters or the directors, officers, controlling persons or affiliates referred to in Section 7 hereof.

14. Certain Defined Terms. For purposes of this Agreement, (a) except where otherwise expressly provided, the term "affiliate" has the meaning set forth in Rule 405 under the Securities Act; (b) the term "business day" means any day other than a day on which banks are permitted or required to be closed in New York City; and (c) the term "subsidiary" has the meaning set forth in Rule 405 under the Securities Act.

15. Compliance with USA Patriot Act. In accordance with the requirements of the USA Patriot Act (Title III of Pub. L. 107-56 (signed into law October 26, 2001)), the Underwriters are required to obtain, verify and record information that identifies their respective clients, including the Company, which information may include the name and address of their respective clients, as well as other information that will allow the Underwriters to properly identify their respective clients.

16. Miscellaneous.

(a) *Notices.* All notices and other communications hereunder shall be in writing and shall be deemed to have been duly given if mailed or transmitted and confirmed by any standard form of telecommunication. Notices to the Underwriters shall be given:

To the Representatives at:

- J.P. Morgan Securities LLC, 383 Madison Avenue, New York, New York 10179 (fax: (212) 622-8358); Attention: Equity Syndicate Desk.
- Cowen and Company, LLC, 599 Lexington Avenue, 20th Floor, New York, New York 10022 (fax: (646) 562-1249); Attention: Head of Equity Capital Markets
- Credit Suisse Securities (USA) LLC, Eleven Madison Avenue, New York, New York 10010-3629 (fax: (212) 325-4296); Attention: IBCM-Legal

To the Company at: Stoke Therapeutics, Inc., 45 Wiggins Avenue, Bedford, Massachusetts 01730, (fax: (781) 538-5731); Attention: Edward M. Kaye, M.D..

(b) *Governing Law.* This Agreement and any claim, controversy or dispute arising under or related to this Agreement shall be governed by and construed in accordance with the laws of the State of New York.

(c) S*ubmission to Jurisdiction.* The Company hereby submits to the exclusive jurisdiction of the U.S. federal and New York state courts in the Borough of Manhattan in The City of New York in any suit or proceeding arising out of or relating to this Agreement or the transactions contemplated hereby. The Company waives any objection which it may now or hereafter have to the laying of venue of any such suit or proceeding in such courts. The Company agrees that final judgment in any such suit, action or proceeding brought in such court shall be conclusive and binding upon the Company and may be enforced in any court to the jurisdiction of which Company is subject by a suit upon such judgment.

(d) *Waiver of Jury Trial.* Each of the parties hereto hereby waives any right to trial by jury in any suit or proceeding arising out of or relating to this Agreement.

(e) *Recognition of the U.S. Special Resolution Regimes*.

(i) In the event that any Underwriter that is a Covered Entity becomes subject to a proceeding under a U.S. Special Resolution Regime, the transfer from such Underwriter of this Agreement, and any interest and obligation in or under this Agreement, will be effective to the same extent as the transfer would be effective under the U.S. Special Resolution Regime if this Agreement, and any such interest and obligation, were governed by the laws of the United States or a state of the United States.

(ii) In the event that any Underwriter that is a Covered Entity or a BHC Act Affiliate of such Underwriter becomes subject to a proceeding under a U.S. Special Resolution Regime, Default Rights under this Agreement that may be exercised against such Underwriter are permitted to the exercised to no greater extent than such Default Rights could be exercised under the U.S. Special Resolution Regime if this Agreement were governed by the laws of the United States or a state of the United States.

As used in this Section 16(g):

"**BHC Act Affiliate**" has the meaning assigned to the term "affiliate" in, and shall be interpreted in accordance with, 12 U.S.C. § 1841(k).

"**Covered Entity**" means any of the following:

(i) a "**covered entity**" as that term is defined in, and interpreted in accordance with, 12 C.F.R. § 252.82(b);

(ii) a "**covered bank**" as that term is defined in, and interpreted in accordance with, 12 C.F.R. § 47.3(b); or

(iii) a "**covered FSI**" as that term is defined in, and interpreted in accordance with, 12 C.F.R. § 382.2(b).

"**Default Right**" has the meaning assigned to that term in, and shall be interpreted in accordance with, 12 C.F.R. §§ 252.81, 47.2 or 382.1, as applicable.

"**U.S. Special Resolution Regime**" means each of (i) the Federal Deposit Insurance Act and the regulations promulgated thereunder and (ii) Title II of the Dodd-Frank Wall Street Reform and Consumer Protection Act and the regulations promulgated thereunder.

(f) *Counterparts.* This Agreement may be signed in counterparts (which may include counterparts delivered by any standard form of telecommunication), each of which shall be an original and all of which together shall constitute one and the same instrument.

(g) *Amendments or Waivers.* No amendment or waiver of any provision of this Agreement, nor any consent or approval to any departure therefrom, shall in any event be effective unless the same shall be in writing and signed by the parties hereto.

(h) *Headings.* The headings herein are included for convenience of reference only and are not intended to be part of, or to affect the meaning or interpretation of, this Agreement.

If the foregoing is in accordance with your understanding, please indicate your acceptance of this Agreement by signing in the space provided below.

Very truly yours,

STOKE THERAPEUTICS, INC.

By: _____
Name:
Title:

[Signature Page to the Underwriting Agreement]

Accepted: As of the date first written above

J.P. MORGAN SECURITIES LLC
COWEN AND COMPANY, LLC
CREDIT SUISSE SECURITIES (USA) LLC

For themselves and on behalf of the
several Underwriters listed
in Schedule 1 hereto.

J.P. MORGAN SECURITIES LLC

By: _____
Authorized Signatory

Name:
Title:

COWEN AND COMPANY, LLC

By: _____
Authorized Signatory

Name:
Title:

CREDIT SUISSE SECURITIES (USA) LLC

By: _____
Authorized Signatory

Name:
Title:

[Signature Page to the Underwriting Agreement]

Schedule 1

Underwriter	Number of Shares
J.P. Morgan Securities LLC	
Cowen and Company, LLC	
Credit Suisse Securities (USA) LLC	
Canaccord Genuity Inc.	
Total	

Annex A

I. Pricing Information Provided Orally by Underwriters

Underwritten Shares: [—] shares

Option Shares: [—] shares

Public Offering Price Per Share: $[—]

II. Issuer Free Writing Prospectus(es)

None

Annex B

Written Testing-the-Waters Communications

2019 Investor Presentations, dated as of April 2019 and May 2019

Annex C

Pricing Term Sheet

[None]

Exhibit A

EGC – Testing the Waters Authorization

In reliance on Section 5(d) of the Securities Act of 1933, as amended (the "**Act**"), Stoke Therapeutics, Inc. (the "**Issuer**") hereby authorizes J.P. Morgan Securities LLC ("**J.P. Morgan**"), Cowen and Company, LLC ("**Cowen**") and Credit Suisse Securities (USA) LLC ("**Credit Suisse**") and their affiliates and their respective employees, to engage on behalf of the Issuer in oral and written communications with potential investors that are "qualified institutional buyers", as defined in Rule 144A under the Act, or institutions that are "accredited investors", as defined in Regulation D under the Act, to determine whether such investors might have an interest in the Issuer's contemplated initial public offering ("**Testing-the-Waters Communications**"). A "Written Testing-the Waters Communication" means any Testing-the-Waters Communication that is a written communication within the meaning of Rule 405 under the Act.

As previously discussed, it is our and your expectation that, unless otherwise approved by the Issuer and the Representatives, neither the Issuer nor any of the Representatives (nor any of the other underwriters for the proposed transaction) will send or give to any potential investor any Written Testing-the-Waters Communication, other than such Testing-the-Waters Communications that are limited to any one or more statements described in Rule 134 under the Act (whether or not reliance on Rule 134 would otherwise be permitted or available under the Act for such Testing-the-Waters Communications) and/or any customary legal or regulatory legends or disclaimers.

The Issuer represents that it is an "emerging growth company" as defined in Section 2(a)(19) of the Act ("**Emerging Growth Company**") and agrees to promptly notify J.P. Morgan, Cowen and Credit Suisse in writing if the Issuer hereafter ceases to be an Emerging Growth Company while this authorization is in effect. If at any time following the distribution of any Written Testing-the-Waters Communication there occurs an event or development as a result of which such Written Testing-the-Waters Communication included or would include an untrue statement of a material fact or omitted or would omit to state a material fact necessary in order to make the statements therein, in the light of the circumstances existing at that subsequent time, not misleading, the Issuer will promptly notify J.P. Morgan, Cowen and Credit Suisse and will promptly amend or supplement, at its own expense, such Written Testing-the-Waters Communication to eliminate or correct such untrue statement or omission.

Nothing in this authorization is intended to limit or otherwise affect the ability of J.P. Morgan, Cowen and Credit Suisse and their respective affiliates and their respective employees, to engage in communications in which they could otherwise lawfully engage in the absence of this authorization, including, without limitation, any written communication containing only one or more of the statements specified under Rule 134(a) under the Act. This authorization shall remain in effect until the Issuer has provided to J.P. Morgan, Cowen and Credit Suisse a written notice revoking this authorization.

All notices as described herein shall be sent by email to the attention of:

- David Ke at David.Ke@jpmorgan.com
- Mariel Healy at Mariel.Healy@cowen.com
- John Kolz at John.Kolz@credit-suisse.com
- Deanna Kirkpatrick at Deanna.Kirkpatrick@davispolk.com
- Jennifer Ying Lan at Jennifer.Lan@davispolk.com

Exhibit B

Form of Waiver of Lock-up

J.P. Morgan Securities LLC
Cowen and Company, LLC
Credit Suisse Securities (USA) LLC

Stoke Therapeutics, Inc.
Public Offering of Common Stock

[—], 20[—]

[Name and Address of
Officer or Director
Requesting Waiver]

Dear Mr./Ms. [Name]:

This letter is being delivered to you in connection with the offering by Stoke Therapeutics, Inc. (the "**Company**") of [—] shares of common stock, $[—] par value (the "**Common Stock**"), of the Company and the lock-up letter dated [—], 2019 (the "**Lock-up Letter**"), executed by you in connection with such offering, and your request for a [waiver] [release] dated [—], 20[—], with respect to [—] shares of Common Stock (the "**Shares**").

J.P. Morgan Securities LLC hereby agrees to [waive] [release] the transfer restrictions set forth in the Lock-up Letter, but only with respect to the Shares, effective [—], 20[—]; provided, however, that such [waiver] [release] is conditioned on the Company announcing the impending [waiver] [release] by press release through a major news service at least two business days before effectiveness of such [waiver] [release]. This letter will serve as notice to the Company of the impending [waiver] [release].

Except as expressly [waived] [released] hereby, the Lock-up Letter shall remain in full force and effect.

[Signature Page as Follows]

Yours very truly,

J.P. MORGAN SECURITIES LLC

By: _____
Authorized Signatory

Name:
Title:

cc: Stoke Therapeutics, Inc.

[Signature Page for Waiver of Lock-Up]

Exhibit C

Form of Press Release

Stoke Therapeutics, Inc.
[Date]

Stoke Therapeutics, Inc. (the "**Company**") announced today that J.P. Morgan Securities LLC, a joint book-running manager in the Company's recent public sale of [—] shares of common stock, is [waiving] [releasing] a lock-up restriction with respect to [—] shares of the Company's common stock held by [certain officers or directors] [an officer or director] of the Company. The [waiver] [release] will take effect on [—], 20[—], and the shares may be sold on or after such date.

This press release is not an offer for sale of the securities in the United States or in any other jurisdiction where such offer is prohibited, and such securities may not be offered or sold in the United States absent registration or an exemption from registration under the United States Securities Act of 1933, as amended.

Exhibit D

Form of Lock-up Agreement

_____, 2019

J.P. Morgan Securities LLC
Cowen and Company, LLC
Credit Suisse Securities (USA) LLC

As Representatives of the
several Underwriters listed
in Schedule 1 hereto

c/o J.P. Morgan Securities LLC
383 Madison Avenue
New York, New York 10179

c/o Cowen and Company, LLC
599 Lexington Avenue
New York, New York 10022

c/o Credit Suisse Securities (USA) LLC
11 Madison Avenue
New York, New York 10010

Re: Stoke Therapeutics, Inc. — Public Offering

Ladies and Gentlemen:

The undersigned understands that you, as Representatives of the several Underwriters, propose to enter into an underwriting agreement (the "**Underwriting Agreement**") with Stoke Therapeutics, Inc., a Delaware corporation (the "**Company**"), providing for the public offering (the "**Public Offering**") by the several Underwriters named in Schedule 1 to the Underwriting Agreement (the "**Underwriters**"), of common stock, $0.0001 per share par value, of the Company (the "**Securities**"). Capitalized terms used herein and not otherwise defined shall have the meanings set forth in the Underwriting Agreement.

In consideration of the Underwriters' agreement to purchase and make the Public Offering of the Securities, and for other good and valuable consideration receipt of which is hereby acknowledged, the undersigned hereby agrees that, without the prior written consent of J.P. Morgan Securities LLC, on behalf of the Underwriters, the undersigned will not, during the period beginning on the date of this letter agreement (this "**Letter Agreement**") and ending 180 days after the date of the prospectus relating to the Public Offering (the "**Prospectus**") (such period, the "**Restricted Period**"), (1) offer, pledge, sell, contract to sell, sell any option or contract to purchase, purchase any option or contract to sell, grant any option, right or warrant to purchase, or otherwise transfer or dispose of, directly or indirectly, any shares of common stock, $0.0001 per share par value, of the Company (the "**Common Stock**") or any securities convertible into or exercisable or exchangeable for Common Stock (including without limitation, Common Stock or such other securities which may be deemed to be beneficially owned by the undersigned in accordance with the rules and regulations of the Securities and Exchange Commission and securities which may be issued upon exercise of a stock option or warrant, collectively the "**Undersigned's Shares**"), or publicly disclose the intention to make any offer, sale, pledge or disposition, (2) enter into

any swap or other agreement that transfers, in whole or in part, any of the economic consequences of ownership of the Undersigned's Shares, whether any such transaction described in clause (1) or (2) above is to be settled by delivery of Common Stock or such other securities, in cash or otherwise or (3) make any demand for or exercise any right with respect to the registration of any shares of Common Stock or any security convertible into or exercisable or exchangeable for Common Stock, in each case other than (A) the Securities to be sold by the undersigned pursuant to the Underwriting Agreement, (B) transfers of the Undersigned's Shares as a bona fide gift or gifts, including bona fide gifts to a charity or educational institution, or for bona fide estate planning purposes, (C) transfers or dispositions of the Undersigned's Shares to any trust for the direct or indirect benefit of the undersigned or the immediate family of the undersigned, (D) transfers or dispositions of the Undersigned's Shares to any corporation, partnership, limited liability company or other entity all of the beneficial ownership interests of which are held by the undersigned or the immediate family of the undersigned, (E) transfers or dispositions of the Undersigned's Shares by will, other testamentary document or intestate succession to the legal representative, heir, beneficiary or a member of the immediate family of the undersigned, (F) distributions of the Undersigned's Shares to partners, members or stockholders of the undersigned, (G) transfers to the undersigned's affiliates or to any investment fund or other entity controlled or managed by, controlling or managing, or under common control with, the undersigned, and (H) transfers pursuant to a *bona fide* third party tender offer, merger, consolidation or other similar transaction made to all holders of the Common Stock and involving a Change of Control of the Company, provided that in the event that the tender offer, merger, consolidation or other such transaction is not completed, the Common Stock owned by the undersigned shall remain subject to the restrictions contained in this Letter Agreement; provided that in the case of any transfer or distribution pursuant to clause (B), (C), (D), (E), (F) or (G), each transferee, donee or distributee shall execute and deliver to the Representatives a lock-up letter in the form of this Letter Agreement; and provided, further, that in the case of any transfer, disposition or distribution pursuant to clause (B), (C), (D), (E), (F) or (G), no filing by any party (donor, donee, transferor or transferee) under the Securities Exchange Act of 1934, as amended (the "**Exchange Act**"), or other public announcement shall be required or shall be made voluntarily in connection with such transfer or distribution (other than a filing on a Form 5 made after the expiration of the Restricted Period referred to above and any such filing shall clearly indicate in the footnotes thereto that the filing relates to the circumstances described in (B), (C), (D), (E), (F) or (G), above, as the case may be, or the filing of a required Schedule 13F or 13G) and any such transfer or distribution shall not involve a disposition for value. If the undersigned is an officer or director of the Company, the undersigned further agrees that the foregoing provisions shall be equally applicable to any Company-directed Securities the undersigned may purchase in the Public Offering. For purposes of this Letter Agreement, "immediate family" shall mean a spouse or domestic partner, child, grandchild or other lineal descendant (including by adoption), father, mother, brother or sister of the undersigned; and "affiliate" shall have the meaning set forth in Rule 405 under the Securities Act of 1933, as amended. For the purposes of clause (H), "Change of Control" shall mean the transfer (whether by tender offer, merger, consolidation or other similar transaction), in one transaction or a series of related transactions, to a person or group of affiliated persons (other than an Underwriter pursuant to the Public Offering), of the Company's voting securities if, after such transfer such person or group of affiliated persons, other than the Company or its subsidiaries, becomes the beneficial owner (as defined in Rules 13d-3 and 13d-5 of the Exchange Act) of 90% or more of the outstanding voting securities of the Company (or the surviving entity).

Furthermore, notwithstanding the restrictions imposed by this Letter Agreement, the undersigned may, without the prior written consent of the Representatives (i) exercise any outstanding warrant, or any option to purchase shares of Common Stock granted under any stock incentive plan or stock purchase plan of the Company, with such plan as disclosed in the Prospectus, provided that the underlying shares of Common Stock shall continue to be subject to the restrictions on transfer set forth in this Letter Agreement, (ii) establish a trading plan pursuant to Rule 10b5-1 under the Exchange Act for the transfer of Common Stock, provided that such plan does not provide for any transfers of Common Stock during the

Restricted Period, and provided, further, that no filing under the Exchange Act or other public announcement shall be required or shall be made voluntarily in connection therewith during the Restricted Period, (iii) transfer or dispose of shares of Common Stock acquired in the Public Offering or on the open market following the Public Offering, provided that no filing under the Exchange Act or other public announcement shall be required or shall be made voluntarily in connection with such transfer or disposition during the Restricted Period (other than a required filing on a Schedule 13F or 13G), (iv) transfer or surrender to the Company shares of Common Stock pursuant to any contractual arrangement that provides the Company with an option to repurchase such shares of Common Stock in connection with the termination of the undersigned's employment or other service relationship with the Company, or pursuant to a right of first refusal with respect to transfers of such shares of Common Stock or other securities, or on a cashless or "net exercise" basis or to cover tax withholding obligations of the undersigned in connection with the vesting or exercise of such shares of Common Stock or other securities, provided that any filing under Section 16 of the Exchange Act shall clearly indicate in the footnotes thereto that the filing relates to the circumstances described in this clause (iv) above and no other public announcement shall be required or shall be made voluntarily in connection with such transfer or surrender, and (v) transfer or dispose of Common Stock by operation of law pursuant to a qualified domestic order or in connection with a divorce settlement or other court order, provided that the recipient of such shares of Common Stock shall execute and deliver to the Representatives a lock-up letter in the form of this Letter Agreement, provided, further that any filing under Section 16 of the Exchange Act shall clearly indicate in the footnotes thereto that the filing relates to the circumstances described in this clause (v) above and no other public announcement shall be required or shall be made voluntarily in connection with such transfer or disposition.

If the undersigned is an officer or director of the Company, (i) J.P. Morgan Securities LLC, on behalf of the Underwriters, agrees that, at least three business days before the effective date of any release or waiver of the foregoing restrictions in connection with a transfer of shares of Common Stock, J.P. Morgan Securities LLC, on behalf of the Underwriters, will notify the Company of the impending release or waiver, and (ii) the Company will agree in the Underwriting Agreement to announce the impending release or waiver by press release through a major news service at least two business days before the effective date of the release or waiver. Any release or waiver granted by J.P. Morgan Securities LLC, on behalf of the Underwriters, hereunder to any such officer or director shall only be effective two business days after the publication date of such press release. The provisions of this paragraph will not apply if (a) the release or waiver is effected solely to permit a transfer not for consideration and (b) the transferee has agreed in writing to be bound by the same terms described in this Letter Agreement to the extent and for the duration that such terms remain in effect at the time of the transfer.

In furtherance of the foregoing, the Company, and any duly appointed transfer agent for the registration or transfer of the securities described herein, are hereby authorized to decline to make any transfer of securities if such transfer would constitute a violation or breach of this Letter Agreement.

The undersigned hereby represents and warrants that the undersigned has full power and authority to enter into this Letter Agreement. All authority herein conferred or agreed to be conferred and any obligations of the undersigned shall be binding upon the successors, assigns, heirs or personal representatives of the undersigned.

The undersigned understands that, if (i) the Company notifies the Representatives in writing prior to the execution of the Underwriting Agreement that it does not intend to proceed with the Public Offering, (ii) the Company files an application with the Securities and Exchange Commission to withdraw the registration statement related to the Public Offering, (iii) the Underwriting Agreement does not become effective by October 31, 2019, or (iv) if the Underwriting Agreement (other than the provisions thereof which survive termination) shall terminate or be terminated prior to payment for and delivery of the Common Stock to be sold thereunder, this Letter Agreement shall automatically, and without any action on the

part of any other party, terminate and be of no further force and effect, and the undersigned shall be released from all obligations under this Letter Agreement. The undersigned understands that the Underwriters are entering into the Underwriting Agreement and proceeding with the Public Offering in reliance upon this Letter Agreement.

The undersigned hereby consents to receipt of this Letter Agreement in electronic form and understands and agrees that this Letter Agreement may be signed electronically. In the event that any signature is delivered by facsimile transmission, electronic mail, or otherwise by electronic transmission evidencing an intent to sign this Letter Agreement, such facsimile transmission, electronic mail or other electronic transmission shall create a valid and binding obligation of the undersigned with the same force and effect as if such signature were an original. Execution and delivery of this Letter Agreement by facsimile transmission, electronic mail or other electronic transmission is legal, valid and binding for all purposes.

This Letter Agreement and any claim, controversy or dispute arising under or related to this Letter Agreement shall be governed by and construed in accordance with the laws of the State of New York.

[Signature Page as Follows]

Very truly yours,

By: _____
Name:
Title:

If signing on behalf of a fund, the name(s) of the fund(s):

[Signature Page to Lock-Up Agreement]

EXHIBIT 3.1

AMENDED AND RESTATED
CERTIFICATE OF INCORPORATION

AMENDED AND RESTATED
CERTIFICATE OF INCORPORATION
OF
STOKE THERAPEUTICS, INC.

(Pursuant to Sections 242 and 245 of the
General Corporation Law of the State of Delaware)

Stoke Therapeutics, Inc., a corporation organized and existing under and by virtue of the provisions of the General Corporation Law of the State of Delaware (the "**General Corporation Law**"),

DOES HEREBY CERTIFY:

1. That the name of this corporation is Stoke Therapeutics, Inc., and that this corporation was originally incorporated pursuant to the General Corporation Law upon the filing of its original Certificate of Incorporation with the Secretary of State of the State of Delaware on June 13, 2014 under the name ASOthera Pharmaceuticals, Inc.

2. That the Board of Directors duly adopted resolutions proposing to amend and restate the Amended and Restated Certificate of Incorporation of this corporation, declaring said amendment and restatement to be advisable and in the best interests of this corporation and its stockholders, and authorizing the appropriate officers of this corporation to solicit the consent of the stockholders therefor, which resolution setting forth the proposed amendment and restatement is as follows:

RESOLVED, that the Amended and Restated Certificate of Incorporation of this corporation be amended and restated in its entirety to read as follows:

FIRST: The name of this corporation is Stoke Therapeutics, Inc. (the "**Corporation**").

SECOND: The address of the registered office of the Corporation in the State of Delaware is 3500 South Dupont Highway, in the City of Dover, County of Kent 19901. The name of its registered agent at such address is Incorporating Services, Ltd.

THIRD: The nature of the business or purposes to be conducted or promoted is to engage in any lawful act or activity for which corporations may be organized under the General Corporation Law.

FOURTH: The total number of shares of all classes of stock which the Corporation shall have authority to issue is (i) 278,527,249 shares of Common Stock, $0.0001 par value per share ("**Common Stock**") and (ii) 225,584,874 shares of Preferred Stock, $0.0001 par value per share ("**Preferred Stock**").

The following is a statement of the designations and the powers, privileges and rights, and the qualifications, limitations or restrictions thereof in respect of each class of capital stock of the Corporation.

A. COMMON STOCK

1. General. The voting, dividend and liquidation rights of the holders of the Common Stock are subject to and qualified by the rights, powers and preferences of the holders of the Preferred Stock set forth herein.

2. Voting. The holders of the Common Stock are entitled to one vote for each share of Common Stock held at all meetings of stockholders (and written actions in lieu of meetings); provided, however, that, except as otherwise required by law, holders of Common Stock, as such, shall not be entitled to vote on any amendment to the certificate of incorporation of the Corporation that relates solely to the terms of one or more outstanding series of Preferred Stock if the holders of such affected series are entitled, either separately or together with the holders of one or more other such series, to vote thereon pursuant to the certificate of incorporation of the Corporation or pursuant to the General Corporation Law. The number of authorized shares of Common Stock may be increased or decreased (but not below the number of shares thereof then outstanding) by (in addition to any vote of the holders of one or more series of Preferred Stock that may be required by the terms of the certificate of incorporation of the Corporation) the affirmative vote of the holders of shares of capital stock of the Corporation representing a majority of the votes represented by all outstanding shares of capital stock of the Corporation entitled to vote, irrespective of the provisions of Section 242(b)(2) of the General Corporation Law.

B. PREFERRED STOCK

49,540,132 shares of the authorized Preferred Stock of the Corporation are designated "**Series A Preferred Stock**," 75,777,370 shares of the authorized Preferred Stock of the Corporation are designated "**Series A-2 Preferred Stock**" and 100,267,372 shares of the authorized Preferred Stock of the corporation are designated "**Series B Preferred Stock**," each with the following rights, preferences, powers, privileges and restrictions, qualifications and limitations. Unless otherwise indicated, references to "sections" or "subsections" in this Part B of this Article Fourth refer to sections and subsections of Part B of this Article Fourth.

1. Dividends.

From and after the date of the issuance of any shares of any series of Preferred Stock, the holders of such shares of Preferred Stock shall be entitled to receive non-cumulative dividends at the rate of eight percent of the Original Issue Price (as defined below) of such series of such Preferred Stock per annum on such shares of Preferred Stock (subject to appropriate adjustment in the event of any stock dividend, stock split, combination or other similar recapitalization with respect to the Preferred Stock) when, as, and if declared by the Board of Directors (the "**Preferred Dividend**").

The Corporation shall not declare, pay or set aside any dividends on shares of any other class or series of capital stock of the Corporation (other than dividends on shares of Common Stock payable in shares of Common Stock) unless (in addition to the obtaining of any consents required elsewhere in the certificate of incorporation of the Corporation) the holders of the Preferred Stock then outstanding shall first receive, or simultaneously receive, a dividend on each outstanding share of Preferred Stock in an amount at least equal to the sum of (i) the amount of the Preferred Dividend then accrued on such share of Preferred Stock for the calendar year in which such dividends are being paid hereunder and not previously paid and (ii) (A) in the case of a dividend on Common Stock or any class or series that is convertible into Common Stock, that dividend per share of Preferred Stock as would equal the product of (1) the dividend payable on each share of such class or series determined, if applicable, as if all shares of such class or series had been converted into Common Stock and (2) the number of shares of Common Stock issuable upon conversion of a share of such Preferred Stock, in each case calculated on the record date for determination of holders entitled to receive such dividend or (B) in the case of a dividend on any class or series that is not convertible into Common Stock, at a rate per share of Preferred Stock determined by (1) dividing the amount of the dividend payable on each share of such class or series of capital stock by the original issuance price of such class or series of capital stock (subject to appropriate adjustment in the event of any stock dividend, stock split, combination or other similar recapitalization with respect to such class or series) and (2) multiplying such fraction by an amount equal to the Original Issue Price (as defined below) of such Preferred Stock; provided that, if the Corporation declares, pays or sets aside, on

the same date, a dividend on shares of more than one class or series of capital stock of the Corporation, the dividend payable to the holders of a series of Preferred Stock pursuant to this Section 1 shall be calculated based upon the dividend on the class or series of capital stock that would result in the highest dividend to such series of Preferred Stock. The " **Original Issue Price**" shall mean $0.8976 per share of Series B Preferred Stock, subject to appropriate adjustment in the event of any stock dividend, stock split, combination or other similar recapitalization with respect to the Series B Preferred Stock, $0.2392 per share of Series A Preferred Stock, subject to appropriate adjustment in the event of any stock dividend, stock split, combination or other similar recapitalization with respect to the Series A Preferred Stock, and $0.3827 per share of Series A-2 Preferred Stock, subject to appropriate adjustment in the event of any stock dividend, stock split, combination or other similar recapitalization with respect to the Series A-2 Preferred Stock.

2. Liquidation, Dissolution or Winding Up; Certain Mergers, Consolidations and Asset Sales.

2.1 Preferential Payments to Holders of Preferred Stock. In the event of any voluntary or involuntary liquidation, dissolution or winding up of the Corporation or Deemed Liquidation Event, the holders of shares of Preferred Stock then outstanding shall be entitled to be paid out of the assets of the Corporation available for distribution to its stockholders before any payment shall be made to the holders of Common Stock by reason of their ownership thereof, an amount per share equal to the Original Issue Price of such share of Preferred Stock, plus any dividends declared but unpaid thereon. If upon any such liquidation, dissolution or winding up of the Corporation or Deemed Liquidation Event, the assets of the Corporation available for distribution to its stockholders shall be insufficient to pay the holders of shares of Preferred Stock the full amount to which they shall be entitled under this Subsection 2.1, the holders of shares of Preferred Stock shall share ratably and on a pari passu basis in any distribution of the assets available for distribution in proportion to the respective amounts which would otherwise be payable in respect of the shares held by them upon such distribution if all amounts payable on or with respect to such shares were paid in full.

2.2 Distribution of Remaining Assets. In the event of any voluntary or involuntary liquidation, dissolution or winding up of the Corporation or Deemed Liquidation Event, after the payment of all preferential amounts required to be paid to the holders of shares of Preferred Stock the remaining assets of the Corporation available for distribution to its stockholders shall be distributed among the holders of the shares of Preferred Stock and Common Stock, pro rata based on the number of shares held by each such holder, treating for this purpose all such securities as if they had been converted to Common Stock pursuant to the terms of the certificate of incorporation of the Corporation immediately prior to such liquidation, dissolution or winding up of the Corporation. The aggregate amount which a holder of a share of Preferred Stock is entitled to receive under Subsections 2.1 and 2.2 is hereinafter referred to as the "**Preferred Stock Liquidation Amount**."

2.3 Deemed Liquidation Events.

2.3.1 Definition. Each of the following events shall be considered a "**Deemed Liquidation Event**" unless the holders of (i) a majority of the then-outstanding shares of Series A Preferred Stock and Series A-2 Preferred Stock, voting as a single class and (ii) a majority of the outstanding Series B Preferred Stock voting together as a single class (clauses (i) and (ii) collectively, the "**Requisite Holders**") elect otherwise by written notice sent to the Corporation at least 10 days prior to the effective date of any such event:

(a) a merger or consolidation in which

(i) the Corporation is a constituent party or

(ii) a subsidiary of the Corporation is a constituent party and the Corporation issues shares of its capital stock pursuant to such merger or consolidation,

except any such merger or consolidation involving the Corporation or a subsidiary in which the shares of capital stock of the Corporation outstanding immediately prior to such merger or consolidation continue to represent, or are converted into or exchanged for shares of capital stock that represent, immediately following such merger or consolidation, at least a majority, by voting power, of the capital stock of (1) the surviving or resulting corporation; or (2) if the surviving or resulting corporation is a wholly owned subsidiary of another corporation immediately following such merger or consolidation, the parent corporation of such surviving or resulting corporation; or

(b) the sale, lease, transfer, exclusive license or other disposition, in a single transaction or series of related transactions, by the Corporation or any subsidiary of the Corporation of all or substantially all the assets of the Corporation and its subsidiaries taken as a whole, or the sale or disposition (whether by merger, consolidation or otherwise) of one or more subsidiaries of the Corporation if substantially all of the assets of the Corporation and its subsidiaries taken as a whole are held by such subsidiary or subsidiaries, except where such sale, lease, transfer, exclusive license or other disposition is to a wholly owned subsidiary of the Corporation.

2.3.2 Effecting a Deemed Liquidation Event.

(a) The Corporation shall not have the power to effect a Deemed Liquidation Event referred to in Subsection 2.3.1(a)(i) unless the agreement or plan of merger or consolidation for such transaction (the "**Merger Agreement**") provides that the consideration payable to the stockholders of the Corporation shall be allocated among the holders of capital stock of the Corporation in accordance with Subsections 2.1 and 2.2.

(b) In the event of a Deemed Liquidation Event referred to in Subsection 2.3.1(a)(ii) or 2.3.1(b), if the Corporation does not effect a dissolution of the Corporation under the General Corporation Law within 90 days after such Deemed Liquidation Event, then (i) the Corporation shall send a written notice to each holder of Preferred Stock no later than the 90th day after the Deemed Liquidation Event advising such holders of their right (and the requirements to be met to secure such right) pursuant to the terms of the following clause; (ii) to require the redemption of such shares of Preferred Stock, and (iii) if the holders of at least a majority of the then outstanding shares of Preferred Stock so request in a written instrument delivered to the Corporation not later than 120 days after such Deemed Liquidation Event, the Corporation shall use the consideration received by the Corporation for such Deemed Liquidation Event (net of any retained liabilities associated with the assets sold or technology licensed, as determined in good faith by the Board of Directors of the Corporation)**,** together with any other assets of the Corporation available for distribution to its stockholders, all to the extent permitted by Delaware law governing distributions to stockholders (the "**Available Proceeds**"), on the 150th day after such Deemed Liquidation Event, to redeem each outstanding share of Preferred Stock at a price per share equal to the respective Preferred Stock Liquidation Amount of such share of Preferred Stock. Notwithstanding the foregoing, in the event of a redemption pursuant to the preceding sentence, if the Available Proceeds are not sufficient to redeem all outstanding shares of Preferred Stock, the Corporation shall ratably and on a pari passu basis redeem each holder's shares of Preferred Stock to the fullest extent of such Available Proceeds, and shall redeem the remaining shares as soon as it may lawfully do so under Delaware law governing distributions to stockholders. Prior to the distribution or redemption provided for in this Subsection 2.3.2(b), the Corporation shall not expend or dissipate the consideration received for such Deemed Liquidation Event, except to discharge expenses incurred in connection with such Deemed Liquidation Event or in the ordinary course of business.

2.3.3 Amount Deemed Paid or Distributed. The amount deemed paid or distributed to the holders of capital stock of the Corporation upon any such merger, consolidation, sale, transfer, exclusive license, other disposition or redemption shall be the cash or the value of the property, rights or securities paid or distributed to such holders by the Corporation or the acquiring person, firm or other entity. The value of such property, rights or securities shall be determined in good faith by the Board of Directors of the Corporation.

2.3.4 Allocation of Escrow and Contingent Consideration. In the event of a Deemed Liquidation Event pursuant to Subsection 2.3.1(a)(i), if any portion of the consideration payable to the stockholders of the Corporation is payable only upon satisfaction of contingencies (the "**Additional Consideration**"), the Merger Agreement shall provide that (a) the portion of such consideration that is not Additional Consideration (such portion, the "**Initial Consideration**") shall be allocated among the holders of capital stock of the Corporation in accordance with Subsections 2.1 and 2.2 as if the Initial Consideration were the only consideration payable in connection with such Deemed Liquidation Event; and (b) any Additional Consideration which becomes payable to the stockholders of the Corporation upon satisfaction of such contingencies shall be allocated among the holders of capital stock of the Corporation in accordance with Subsections 2.1 and 2.2 after taking into account the previous payment of the Initial Consideration as part of the same transaction. For the purposes of this Subsection 2.3.4, consideration placed into escrow or retained as holdback to be available for satisfaction of indemnification or similar obligations in connection with such Deemed Liquidation Event shall be deemed to be Additional Consideration.

3. Voting.

3.1 General. On any matter presented to the stockholders of the Corporation for their action or consideration at any meeting of stockholders of the Corporation (or by written consent of stockholders in lieu of meeting), each holder of outstanding shares of Preferred Stock shall be entitled to cast the number of votes equal to the number of whole shares of Common Stock into which the shares of Preferred Stock held by such holder are convertible as of the record date for determining stockholders entitled to vote on such matter. Except as provided by law or by the other provisions of the certificate of incorporation of the Corporation, holders of Preferred Stock shall vote together with the holders of Common Stock as a single class.

3.2 Election of Directors.

3.2.1 General. At any meeting held for the purpose of electing a director, the presence in person or by proxy of the holders of a majority of the outstanding shares of the class or series entitled to elect such director shall constitute a quorum for the purpose of electing such director. Except as otherwise provided in this Subsection 3.2, a vacancy in any directorship filled by the holders of any class or series shall be filled only by vote or written consent in lieu of a meeting of the holders of such class or series or by any remaining director or directors elected by the holders of such class or series pursuant to this Subsection 3.2.

3.2.2 Common Directors. The holders of record of the shares of Common Stock, exclusively and as a separate class, shall be entitled to elect two directors of the Corporation (the "**Common Directors**"). If the holders of shares of Common Stock fail to elect a sufficient number of directors to fill all directorships for which they are entitled to elect directors, voting exclusively and as a separate class, pursuant to the first sentence of this Subsection 3.2.2, then any directorship not so filled shall remain vacant until such time as the holders of the Common Stock elect a person to fill such directorship by vote or written consent in lieu of a meeting; and no such directorship may be filled by stockholders of the Corporation other than by the stockholders of the Corporation that are entitled to elect a person to fill such directorship, voting exclusively and as a separate class.

3.2.3 Preferred Directors. For so long as any shares of Preferred Stock remain outstanding, the holders of record of the shares of Preferred Stock, exclusively and as a separate class, shall be entitled to elect two directors of the Corporation (the "**Preferred Directors**"). If the holders of shares of Preferred Stock fail to elect a sufficient number of directors to fill all directorships for which they are entitled to elect directors, voting exclusively and as a separate class, pursuant to the first sentence of this Subsection 3.2.3, then any directorship not so filled shall remain vacant until such time as the holders of the Preferred Stock elect a person to fill such directorship by vote or written consent in lieu of a meeting; and no such directorship may be filled by stockholders of the Corporation other than by the stockholders of the Corporation that are entitled to elect a person to fill such directorship, voting exclusively and as a separate class.

3.2.4 Remaining Directors. The holders of the Common Stock and the Preferred Stock, voting together as a single class on an as-if-converted basis, shall be entitled to elect all remaining directors at each meeting or pursuant to each consent of the Company's stockholders for the election of directors (the "**Remaining Directors**"). Notwithstanding anything to the contrary provided herein, if any vacancy in the office of any Remaining Director exists, such vacancy may be filled (either contingently or otherwise) by the stockholders as specified in this Section 3.2.4 or by at least a majority of the members of the Board then in office, although less than a quorum, or by a sole remaining member of the Board then in office, even if such directors or such sole remaining director were not elected by the holders of the class, classes or series that are entitled to elect a director or directors to office under the provisions of this Section 3.2.4 and such electing director or directors shall specify at the time of such election the specific vacant directorship being filled.

3.2.5 Removal of Directors. Any director elected as provided in the Subsections 3.2.1, 3.2.2 or 3.3.3 may be removed without cause by, and only by, the affirmative vote of the holders of the shares of the class or series of capital stock entitled to elect such director or directors, given either at a special meeting of such stockholders duly called for that purpose or pursuant to a written consent of stockholders.

3.3 Preferred Stock Protective Provisions. For so long as any shares of Preferred Stock remain outstanding, the Corporation shall not, either directly or indirectly by amendment, merger, consolidation or otherwise, do any of the following without (in addition to any other vote required by law or the certificate of incorporation of the Corporation) the written consent or affirmative vote of the Requisite Holders, given in writing or by vote at a meeting, and any such act or transaction entered into without such consent or vote shall be null and void *ab initio*, and of no force or effect.

3.3.1 liquidate, dissolve or wind-up the business and affairs of the Corporation, effect any merger or consolidation, any asset transfer, or any license of intellectual property out of the ordinary course of business, any acquisition or any other Deemed Liquidation Event, or consent to any of the foregoing;

3.3.2 amend, alter or repeal any provision of the certificate of incorporation or bylaws of the Corporation;

3.3.3 create, or authorize the creation of, or issue or obligate itself to issue shares of, any additional class or series of capital stock unless the same ranks junior to the Preferred Stock with respect to Liquidation Preference, the payment of dividends, rights of redemption and voting, or increase the authorized number of shares of Preferred Stock or increase the authorized number of shares of any additional class or series of capital stock unless the same ranks junior to the Preferred Stock with respect to Liquidation Preference, the payment of dividends, rights of redemption and voting;

3.3.4 (i) reclassify, alter or amend any existing security of the Corporation that is pari passu with the Preferred Stock in respect of the distribution of assets on the liquidation, dissolution or winding up of the Corporation, the payment of dividends or rights of redemption, if such reclassification, alteration or amendment would render such other security senior to the Preferred Stock in respect of any such right, preference, or privilege or (ii) reclassify, alter or amend any existing security of the Corporation that is junior to the Preferred Stock in respect of the distribution of assets on the liquidation, dissolution or winding up of the Corporation, the payment of dividends or rights of redemption, if such reclassification, alteration or amendment would render such other security senior to or pari passu with the Preferred Stock in respect of any such right, preference or privilege;

3.3.5 purchase or redeem (or permit any subsidiary to purchase or redeem) or pay or declare any dividend or make any distribution on, any shares of capital stock of the Corporation other than (i) redemptions of or dividends or distributions on the Preferred Stock as expressly authorized herein and (ii) repurchases of stock from former employees, officers, directors, consultants or other persons who performed services for the Corporation or any subsidiary in connection with the cessation of such employment or service at the lower of the original purchase price or the then-current fair market value thereof;

3.3.6 create, or authorize the creation of, or issue, or authorize the issuance of any debt security, or permit any subsidiary to take any such action with respect to any debt security, if the aggregate indebtedness of the Corporation and its subsidiaries for borrowed money following such action would exceed $100,000 unless such debt security has received the prior approval of the Board of Directors;

3.3.7 increase the authorized number of shares of Preferred Stock or Common Stock;

3.3.8 increase or decrease the authorized number of or method of selecting directors constituting the Board of Directors; or

3.3.9 enter into any interested party transaction (other than transactions that (i) involve payment obligations of, or payments to, the Corporation of immaterial amounts or (ii) do not involve any exchange of cash or other property, in each case, in the ordinary course of business), unless approved by the Board of Directors (including a disinterested majority of directors).

4. Optional Conversion.

The holders of the Preferred Stock shall have conversion rights as follows (the "**Conversion Rights**"):

4.1 Right to Convert.

4.1.1 Conversion Ratio. Each share of Preferred Stock shall be convertible, at the option of the holder thereof, at any time and from time to time, and without the payment of additional consideration by the holder thereof, into such number of fully paid and non-assessable shares of Common Stock as is determined by dividing the Original Issue Price of such share of Preferred Stock by the Conversion Price (as defined below) of such share of Preferred Stock in effect at the time of conversion. The "**Conversion Price**" for each share of Preferred Stock shall initially be equal to the Original Issue Price of such share of Preferred Stock; for the sake of clarity, any reference to "Conversion Price" herein refers to the respective Conversion Price of each series of Preferred Stock. Such initial Conversion Price, and the rate at which shares of Preferred Stock may be converted into shares of Common Stock, shall be subject to adjustment as provided below.

4.1.2 Termination of Conversion Rights. In the event of a liquidation, dissolution or winding up of the Corporation or a Deemed Liquidation Event, the Conversion Rights shall terminate at the close of business on the last full day preceding the date fixed for the payment of any such amounts distributable on such event to the holders of Preferred Stock.

4.2 Fractional Shares. No fractional shares of Common Stock shall be issued upon conversion of the Preferred Stock. In lieu of any fractional shares to which the holder would otherwise be entitled, the Corporation shall pay cash equal to such fraction multiplied by the fair market value of a share of Common Stock as determined in good faith by the Board of Directors of the Corporation. Whether or not fractional shares would be issuable upon such conversion shall be determined on the basis of the total number of shares of Preferred Stock the holder is at the time converting into Common Stock and the aggregate number of shares of Common Stock issuable upon such conversion.

4.3 Mechanics of Conversion.

4.3.1 Notice of Conversion. In order for a holder of Preferred Stock to voluntarily convert shares of Preferred Stock into shares of Common Stock, such holder shall (a) provide written notice to the Corporation's transfer agent at the office of the transfer agent for the Preferred Stock (or at the principal office of the Corporation if the Corporation serves as its own transfer agent) that such holder elects to convert all or any number of such holder's shares of Preferred Stock and, if applicable, any event on which such conversion is contingent and (b), if such holder's shares are certificated, surrender the certificate or certificates for such shares of Preferred Stock (or, if such registered holder alleges that such certificate has been lost, stolen or destroyed, a lost certificate affidavit and agreement reasonably acceptable to the Corporation to indemnify the Corporation against any claim that may be made against the Corporation on account of the alleged loss, theft or destruction of such certificate), at the office of the transfer agent for the Preferred Stock (or at the principal office of the Corporation if the Corporation serves as its own transfer agent). Such notice shall state such holder's name or the names of the nominees in which such holder wishes the shares of Common Stock to be issued. If required by the Corporation, any certificates surrendered for conversion shall be endorsed or accompanied by a written instrument or instruments of transfer, in form satisfactory to the Corporation, duly executed by the registered holder or his, her or its attorney duly authorized in writing. The close of business on the date of receipt by the transfer agent (or by the Corporation if the Corporation serves as its own transfer agent) of such notice and, if applicable, certificates (or lost certificate affidavit and agreement) shall be the time of conversion (the "**Conversion Time**"), and the shares of Common Stock issuable upon conversion of the specified shares shall be deemed to be outstanding of record as of such date. The Corporation shall, as soon as practicable after the Conversion Time (i) issue and deliver to such holder of Preferred Stock, or to his, her or its nominees, a certificate or certificates for the number of full shares of Common Stock issuable upon such conversion in accordance with the provisions hereof and a certificate for the number (if any) of the shares of Preferred Stock represented by the surrendered certificate that were not converted into Common Stock, (ii) pay in cash such amount as provided in Subsection 4.2 in lieu of any fraction of a share of Common Stock otherwise issuable upon such conversion and (iii) pay all declared but unpaid dividends on the shares of Preferred Stock converted.

4.3.2 Reservation of Shares. The Corporation shall at all times when the Preferred Stock shall be outstanding, reserve and keep available out of its authorized but unissued capital stock, for the purpose of effecting the conversion of the Preferred Stock, such number of its duly authorized shares of Common Stock as shall from time to time be sufficient to effect the conversion of all outstanding Preferred Stock; and if at any time the number of authorized but unissued shares of Common Stock shall not be sufficient to effect the conversion of all then outstanding shares of the Preferred Stock, the Corporation shall take such corporate action as may be necessary to increase its authorized but unissued shares of Common Stock to such number of shares as shall be sufficient for such purposes,

including, without limitation, engaging in best efforts to obtain the requisite stockholder approval of any necessary amendment to the certificate of incorporation of the Corporation. Before taking any action which would cause an adjustment reducing the Conversion Price below the then par value of the shares of Common Stock issuable upon conversion of the Preferred Stock, the Corporation will take any corporate action which may, in the opinion of its counsel, be necessary in order that the Corporation may validly and legally issue fully paid and non-assessable shares of Common Stock at such adjusted Conversion Price.

4.3.3 Effect of Conversion. All shares of Preferred Stock which shall have been surrendered for conversion as herein provided shall no longer be deemed to be outstanding and all rights with respect to such shares shall immediately cease and terminate at the Conversion Time, except only the right of the holders thereof to receive shares of Common Stock in exchange therefor, to receive payment in lieu of any fraction of a share otherwise issuable upon such conversion as provided in Subsection 4.2 and to receive payment of any dividends declared but unpaid thereon. Any shares of Preferred Stock so converted shall be retired and cancelled and may not be reissued as shares of Preferred Stock, and the Corporation may thereafter take such appropriate action (without the need for stockholder action) as may be necessary to reduce the authorized number of shares of Preferred Stock and the authorized number of shares of the applicable series of Preferred Stock accordingly.

4.3.4 No Further Adjustment. Upon any such conversion, no adjustment to the Conversion Price shall be made for any declared but unpaid dividends on the Preferred Stock surrendered for conversion or on the Common Stock delivered upon conversion.

4.3.5 Taxes. The Corporation shall pay any and all issue and other similar taxes that may be payable in respect of any issuance or delivery of shares of Common Stock upon conversion of shares of Preferred Stock pursuant to this Section 4. The Corporation shall not, however, be required to pay any tax which may be payable in respect of any transfer involved in the issuance and delivery of shares of Common Stock in a name other than that in which the shares of Preferred Stock so converted were registered, and no such issuance or delivery shall be made unless and until the person or entity requesting such issuance has paid to the Corporation the amount of any such tax or has established, to the satisfaction of the Corporation, that such tax has been paid.

4.4 Adjustments to Conversion Price for Diluting Issues.

4.4.1 Special Definitions. For purposes of this Article Fourth, the following definitions shall apply:

(a) "**Option**" shall mean rights, options or warrants to subscribe for, purchase or otherwise acquire Common Stock or Convertible Securities.

(b) "**Original Issue Date**" shall mean the date on which the first share of Series B Preferred Stock was issued.

(c) "**Convertible Securities**" shall mean any evidences of indebtedness, shares or other securities directly or indirectly convertible into or exchangeable for Common Stock, but excluding Options.

(d) "**Additional Shares of Common Stock**" shall mean all shares of Common Stock issued (or, pursuant to Subsection 4.4.3 below, deemed to be issued) by the Corporation after the Original Issue Date, other than (1) the following shares of Common Stock and (2) shares of Common Stock deemed issued pursuant to the following Options and Convertible Securities (clauses (1) and (2), collectively, "**Exempted Securities**"):

(i) shares of Common Stock, Options or Convertible Securities issued as a dividend or distribution on Preferred Stock;

(ii) shares of Common Stock, Options or Convertible Securities issued by reason of a dividend, stock split, split-up or other distribution on shares of Common Stock that is covered by Subsection 4.5, 4.6, 4.7 or 4.8**;**

(iii) shares of Common Stock or Options issued to employees or directors of, or consultants or advisors to, the Corporation or any of its subsidiaries pursuant to a plan, agreement or arrangement approved by the Board of Directors of the Corporation, including the Preferred Directors;

(iv) shares of Common Stock or Convertible Securities actually issued upon the exercise of Options or shares of Common Stock actually issued upon the conversion or exchange of Convertible Securities, in each case provided such issuance is pursuant to the terms of such Option or Convertible Security;

(v) shares of Common Stock, Options or Convertible Securities issued to banks, equipment lessors or other financial institutions, or to real property lessors, pursuant to a debt financing, equipment leasing or real property leasing transaction approved by the Board of Directors of the Corporation, including the Preferred Directors;

(vi) shares of Common Stock, Options or Convertible Securities issued to suppliers or third party service providers in connection with the provision of goods or services pursuant to transactions approved by the Board of Directors of the Corporation, including the Preferred Directors;

(vii) shares of Common Stock, Options or Convertible Securities issued in to entities in connection with joint ventures, development projects, acquisitions, or other strategic transaction, provided that such issuances are approved by the Board of Directors of the Corporation, including the Preferred Directors;

(viii) shares of Common Stock issued upon the closing of a firmly underwritten public offering of shares of Common Stock of the Company pursuant to a registration statement under the Securities Act of 1933 at a pre-money valuation of at least $275,000,000 and for a total offering of not less than $75,000,000 (before deduction of underwriters commissions and expenses) (a "**Qualified IPO**"); or

(ix) any other shares of Common Stock, Options or Convertible Securities issued upon the written consent of the holders of at least a majority of the then outstanding shares of Preferred Stock, voting together as a single class on an as-converted basis, that such shares of Common Stock, Options or Convertible Securities shall not constitute Additional Shares of Common Stock.

4.4.2 No Adjustment of Conversion Price. No adjustment in the Conversion Price of a particular series of Preferred Stock shall be made as the result of the issuance or deemed issuance of Additional Shares of Common Stock if the Corporation receives written notice from the holders of at least a majority of the then outstanding shares of such series of Preferred Stock agreeing that no such adjustment shall be made as the result of the issuance or deemed issuance of such Additional Shares of Common Stock.

4.4.3 Deemed Issue of Additional Shares of Common Stock.

(a) If the Corporation at any time or from time to time after the Original Issue Date shall issue any Options or Convertible Securities (excluding Options or Convertible Securities which are themselves Exempted Securities) or shall fix a record date for the determination of holders of any class of securities entitled to receive any such Options or Convertible Securities, then the maximum number of shares of Common Stock (as set forth in the instrument relating thereto, assuming the satisfaction of any conditions to exercisability, convertibility or exchangeability but without regard to any provision contained therein for a subsequent adjustment of such number) issuable upon the exercise of such Options or, in the case of Convertible Securities and Options therefor, the conversion or exchange of such Convertible Securities, shall be deemed to be Additional Shares of Common Stock issued as of the time of such issue or, in case such a record date shall have been fixed, as of the close of business on such record date.

(b) If the terms of any Option or Convertible Security, the issuance of which resulted in an adjustment to the Conversion Price pursuant to the terms of Subsection 4.4.4, are revised as a result of an amendment to such terms or any other adjustment pursuant to the provisions of such Option or Convertible Security (but excluding automatic adjustments to such terms pursuant to anti-dilution or similar provisions of such Option or Convertible Security) to provide for either (1) any increase or decrease in the number of shares of Common Stock issuable upon the exercise, conversion and/or exchange of any such Option or Convertible Security or (2) any increase or decrease in the consideration payable to the Corporation upon such exercise, conversion and/or exchange, then, effective upon such increase or decrease becoming effective, the Conversion Price computed upon the original issue of such Option or Convertible Security (or upon the occurrence of a record date with respect thereto) shall be readjusted to such Conversion Price as would have obtained had such revised

terms been in effect upon the original date of issuance of such Option or Convertible Security. Notwithstanding the foregoing, no readjustment pursuant to this clause (b) shall have the effect of increasing the Conversion Price to an amount which exceeds the lower of (i) the Conversion Price in effect immediately prior to the original adjustment made as a result of the issuance of such Option or Convertible Security, or (ii) the Conversion Price that would have resulted from any issuances of Additional Shares of Common Stock (other than deemed issuances of Additional Shares of Common Stock as a result of the issuance of such Option or Convertible Security) between the original adjustment date and such readjustment date.

(c) If the terms of any Option or Convertible Security (excluding Options or Convertible Securities which are themselves Exempted Securities), the issuance of which did not result in an adjustment to the Conversion Price pursuant to the terms of Subsection 4.4.4 (either because the consideration per share (determined pursuant to Subsection 4.4.5) of the Additional Shares of Common Stock subject thereto was equal to or greater than the Conversion Price then in effect, or because such Option or Convertible Security was issued before the Original Issue Date), are revised after the Original Issue Date as a result of an amendment to such terms or any other adjustment pursuant to the provisions of such Option or Convertible Security (but excluding automatic adjustments to such terms pursuant to anti-dilution or similar provisions of such Option or Convertible Security) to provide for either (1) any increase in the number of shares of Common Stock issuable upon the exercise, conversion or exchange of any such Option or Convertible Security or (2) any decrease in the consideration payable to the Corporation upon such exercise, conversion or exchange, then such Option or Convertible Security, as so amended or adjusted, and the Additional Shares of Common Stock subject thereto (determined in the manner provided in Subsection 4.4.3(a)) shall be deemed to have been issued effective upon such increase or decrease becoming effective.

(d) Upon the expiration or termination of any unexercised Option or unconverted or unexchanged Convertible Security (or portion thereof) which resulted (either upon its original issuance or upon a revision of its terms) in an adjustment to the Conversion Price pursuant to the terms of Subsection 4.4.4, the Conversion Price shall be readjusted to such Conversion Price as would have obtained had such Option or Convertible Security (or portion thereof) never been issued.

(e) If the number of shares of Common Stock issuable upon the exercise, conversion and/or exchange of any Option or Convertible Security, or the consideration payable to the Corporation upon such exercise, conversion and/or exchange, is calculable at the time such Option or Convertible Security is issued or amended but is subject to adjustment based upon subsequent events, any adjustment to the Conversion Price provided for in this Subsection 4.4.3 shall be effected at the time of such issuance or amendment based on such number of shares or amount of consideration without regard to any provisions for subsequent adjustments (and any subsequent adjustments shall be treated as provided in clauses (b) and (c) of this Subsection 4.4.3). If the number of shares of Common Stock issuable upon the exercise, conversion and/or exchange of any Option or Convertible Security, or the consideration payable to the Corporation upon such exercise, conversion and/or exchange, cannot be calculated at all at the time such Option or Convertible Security is issued or amended, any adjustment to the Conversion Price that would result under the terms of this Subsection 4.4.3 at the time of such issuance or amendment shall instead be effected at the time such number of shares and/or amount of consideration is first calculable (even if subject to subsequent adjustments), assuming for purposes of calculating such adjustment to the Conversion Price that such issuance or amendment took place at the time such calculation can first be made.

4.4.4 Adjustment of Conversion Price Upon Issuance of Additional Shares of Common Stock. In the event the Corporation shall at any time after the Original Issue Date issue Additional Shares of Common Stock (including Additional Shares of Common Stock deemed to be

issued pursuant to Subsection 4.4.3), without consideration or for a consideration per share less than the Conversion Price of any series of Preferred Stock in effect immediately prior to such issue, then such Conversion Price for such series of Preferred Stock shall be reduced, concurrently with such issue, to a price (calculated to the nearest one-hundredth of a cent) determined in accordance with the following formula:

$$CP_2 = CP_1 * (A + B) \div (A + C).$$

For purposes of the foregoing formula, the following definitions shall apply:

(a) "CP_2" shall mean the Conversion Price in effect immediately after such issue of Additional Shares of Common Stock

(b) "CP_1" shall mean the Conversion Price in effect immediately prior to such issue of Additional Shares of Common Stock;

(c) "A" shall mean the number of shares of Common Stock outstanding immediately prior to such issue of Additional Shares of Common Stock (treating for this purpose as outstanding all shares of Common Stock issuable upon exercise of Options outstanding immediately prior to such issue or upon conversion or exchange of Convertible Securities (including the Preferred Stock) outstanding (assuming exercise of any outstanding Options therefor) immediately prior to such issue);

(d) "B" shall mean the number of shares of Common Stock that would have been issued if such Additional Shares of Common Stock had been issued at a price per share equal to CP_1 (determined by dividing the aggregate consideration received by the Corporation in respect of such issue by CP_1); and

(e) "C" shall mean the number of such Additional Shares of Common Stock issued in such transaction.

4.4.5 Determination of Consideration. For purposes of this Subsection 4.4, the consideration received by the Corporation for the issue of any Additional Shares of Common Stock shall be computed as follows:

(a) Cash and Property: Such consideration shall:

(i) insofar as it consists of cash, be computed at the aggregate amount of cash received by the Corporation, excluding amounts paid or payable for accrued interest;

(ii) insofar as it consists of property other than cash, be computed at the fair market value thereof at the time of such issue, as determined in good faith by the Board of Directors of the Corporation; and

(iii) in the event Additional Shares of Common Stock are issued together with other shares or securities or other assets of the Corporation for consideration which covers both, be the

proportion of such consideration so received, computed as provided in clauses (i) and (ii) above, as determined in good faith by the Board of Directors of the Corporation.

(b) Options and Convertible Securities. The consideration per share received by the Corporation for Additional Shares of Common Stock deemed to have been issued pursuant to Subsection 4.4.3, relating to Options and Convertible Securities, shall be determined by dividing:

(i) The total amount, if any, received or receivable by the Corporation as consideration for the issue of such Options or Convertible Securities, plus the minimum aggregate amount of additional consideration (as set forth in the instruments relating thereto, without regard to any provision contained therein for a subsequent adjustment of such consideration) payable to the Corporation upon the exercise of such Options or the conversion or exchange of such Convertible Securities, or in the case of Options for Convertible Securities, the exercise of such Options for Convertible Securities and the conversion or exchange of such Convertible Securities, by

(ii) the maximum number of shares of Common Stock (as set forth in the instruments relating thereto, without regard to any provision contained therein for a subsequent adjustment of such number) issuable upon the exercise of such Options or the conversion or exchange of such Convertible Securities, or in the case of Options for Convertible Securities, the exercise of such Options for Convertible Securities and the conversion or exchange of such Convertible Securities.

4.4.6 Multiple Closing Dates. In the event the Corporation shall issue on more than one date Additional Shares of Common Stock that are a part of one transaction or a series of related transactions and that would result in an adjustment to the Conversion Price pursuant to the terms of Subsection 4.4.4, then, upon the final such issuance, the Conversion Price shall be readjusted to give effect to all such issuances as if they occurred on the date of the first such issuance (and without giving effect to any additional adjustments as a result of any such subsequent issuances within such period).

4.5 Adjustment for Stock Splits and Combinations. If the Corporation shall at any time or from time to time after the Original Issue Date effect a subdivision of the outstanding Common Stock, the Conversion Price in effect immediately before that subdivision shall be proportionately decreased so that the number of shares of Common Stock issuable on conversion of each share of Preferred Stock shall be increased in proportion to such increase in the aggregate number of shares of Common Stock outstanding. If the Corporation shall at any time or from time to time after the Original Issue Date combine the outstanding shares of Common Stock, the Conversion Price in effect

immediately before the combination shall be proportionately increased so that the number of shares of Common Stock issuable on conversion of each share of such series shall be decreased in proportion to such decrease in the aggregate number of shares of Common Stock outstanding. Any adjustment under this subsection shall become effective at the close of business on the date the subdivision or combination becomes effective.

4.6 Adjustment for Certain Dividends and Distributions. In the event the Corporation at any time or from time to time after the Original Issue Date shall make or issue, or fix a record date for the determination of holders of Common Stock entitled to receive, a dividend or other distribution payable on the Common Stock in additional shares of Common Stock, then and in each such event the Conversion Price in effect immediately before such event shall be decreased as of the time of such issuance or, in the event such a record date shall have been fixed, as of the close of business on such record date, by multiplying the Conversion Price then in effect by a fraction:

(1) the numerator of which shall be the total number of shares of Common Stock issued and outstanding immediately prior to the time of such issuance or the close of business on such record date, and

(2) the denominator of which shall be the total number of shares of Common Stock issued and outstanding immediately prior to the time of such issuance or the close of business on such record date plus the number of shares of Common Stock issuable in payment of such dividend or distribution.

Notwithstanding the foregoing (a) if such record date shall have been fixed and such dividend is not fully paid or if such distribution is not fully made on the date fixed therefor, the Conversion Price shall be recomputed accordingly as of the close of business on such record date and thereafter the Conversion Price shall be adjusted pursuant to this subsection as of the time of actual payment of such dividends or distributions; and (b) that no such adjustment shall be made if the holders of Preferred Stock simultaneously receive a dividend or other distribution of shares of Common Stock in a number equal to the number of shares of Common Stock as they would have received if all outstanding shares of Preferred Stock had been converted into Common Stock on the date of such event.

4.7 Adjustments for Other Dividends and Distributions. In the event the Corporation at any time or from time to time after the Original Issue Date shall make or issue, or fix a record date for the determination of holders of Common Stock entitled to receive, a dividend or other distribution payable in securities of the Corporation (other than a distribution of shares of Common Stock in respect of outstanding shares of Common Stock) or in other property and the provisions of Section 1 do not apply to such dividend or distribution, then and in each such event the holders of Preferred Stock shall receive, simultaneously with the distribution to the holders of Common Stock, a dividend or other distribution of such securities or other property in an amount equal to the amount of such securities or other property as they would have received if all outstanding shares of Preferred Stock had been converted into Common Stock on the date of such event.

4.8 Adjustment for Merger or Reorganization, etc. Subject to the provisions of Subsection 2.3, if there shall occur any reorganization, recapitalization, reclassification, consolidation or merger involving the Corporation in which the Common Stock (but not the Preferred Stock) is converted into or exchanged for securities, cash or other property (other than a transaction covered by Subsections 4.4, 4.6 or 4.7), then, following any such reorganization, recapitalization, reclassification, consolidation or merger, each share of Preferred Stock shall thereafter be convertible in lieu of the Common Stock into which it was convertible prior to such event into the kind and amount of securities, cash or other property which a holder of the number of shares of Common Stock of the Corporation issuable upon conversion of one share of Preferred Stock immediately prior to such reorganization,

recapitalization, reclassification, consolidation or merger would have been entitled to receive pursuant to such transaction; and, in such case, appropriate adjustment (as determined in good faith by the Board of Directors of the Corporation) shall be made in the application of the provisions in this Section 4 with respect to the rights and interests thereafter of the holders of the Preferred Stock, to the end that the provisions set forth in this Section 4 (including provisions with respect to changes in and other adjustments of the Conversion Price) shall thereafter be applicable, as nearly as reasonably may be, in relation to any securities or other property thereafter deliverable upon the conversion of the Preferred Stock. For the avoidance of doubt, nothing in this Subsection 4.8 shall be construed as preventing the holders of Preferred Stock from seeking any appraisal rights to which they are otherwise entitled under the DGCL in connection with a merger triggering an adjustment hereunder, nor shall this Subsection 4.8 be deemed conclusive evidence of the fair value of the shares of Preferred Stock in any such appraisal proceeding.

4.9 Certificate as to Adjustments. Upon the occurrence of each adjustment or readjustment of the Conversion Price pursuant to this Section 4, the Corporation at its expense shall, as promptly as reasonably practicable but in any event not later than 10 days thereafter, compute such adjustment or readjustment in accordance with the terms hereof and furnish to each holder of Preferred Stock a certificate setting forth such adjustment or readjustment (including the kind and amount of securities, cash or other property into which the Preferred Stock is convertible) and showing in detail the facts upon which such adjustment or readjustment is based. The Corporation shall, as promptly as reasonably practicable after the written request at any time of any holder of Preferred Stock (but in any event not later than 10 days thereafter), furnish or cause to be furnished to such holder a certificate setting forth (i) the Conversion Price then in effect, and (ii) the number of shares of Common Stock and the amount, if any, of other securities, cash or property which then would be received upon the conversion of Preferred Stock.

4.10 Notice of Record Date. In the event:

(a) the Corporation shall take a record of the holders of its Common Stock (or other capital stock or securities at the time issuable upon conversion of the Preferred Stock) for the purpose of entitling or enabling them to receive any dividend or other distribution, or to receive any right to subscribe for or purchase any shares of capital stock of any class or any other securities, or to receive any other security; or

(b) of any capital reorganization of the Corporation, any reclassification of the Common Stock of the Corporation, or any Deemed Liquidation Event; or

(c) of the voluntary or involuntary dissolution, liquidation or winding-up of the Corporation,

then, and in each such case, the Corporation will send or cause to be sent to the holders of the Preferred Stock a notice specifying, as the case may be, (i) the record date for such dividend, distribution or right, and the amount and character of such dividend, distribution or right, or (ii) the effective date on which such reorganization, reclassification, consolidation, merger, transfer, dissolution, liquidation or winding-up is proposed to take place, and the time, if any is to be fixed, as of which the holders of record of Common Stock (or such other capital stock or securities at the time issuable upon the conversion of the Preferred Stock) shall be entitled to exchange their shares of Common Stock (or such other capital stock or securities) for securities or other property deliverable upon such reorganization, reclassification, consolidation, merger, transfer, dissolution, liquidation or winding-up, and the amount per share and character of such exchange applicable to the Preferred Stock and the Common Stock. Such notice shall be sent at least 10 days prior to the record date or effective date for the event specified in such notice.

5. Mandatory Conversion.

5.1 Trigger Events. Upon either (a) a Qualified IPO or (b) the date and time, or the occurrence of an event, specified by vote or written consent of the Requisite Holders (the time of such closing or the date and time specified or the time of the event specified in such vote or written consent is referred to herein as the "**Mandatory Conversion Time**"), then (i) all outstanding shares of Preferred Stock shall automatically be converted into shares of Common Stock, at the then effective conversion rate as calculated pursuant to Subsection 4.1.1. and (ii) such shares may not be reissued by the Corporation.

5.2 Procedural Requirements. All holders of record of shares of Preferred Stock shall be sent written notice of the Mandatory Conversion Time and the place designated for mandatory conversion of all such shares of Preferred Stock pursuant to this Section 5. Such notice need not be sent in advance of the occurrence of the Mandatory Conversion Time. Upon receipt of such notice, each holder of shares of Preferred Stock in certificated form shall surrender his, her or its certificate or certificates for all such shares (or, if such holder alleges that such certificate has been lost, stolen or destroyed, a lost certificate affidavit and agreement reasonably acceptable to the Corporation to indemnify the Corporation against any claim that may be made against the Corporation on account of the alleged loss, theft or destruction of such certificate) to the Corporation at the place designated in such notice. If so required by the Corporation, any certificates surrendered for conversion shall be endorsed or accompanied by written instrument or instruments of transfer, in form satisfactory to the Corporation, duly executed by the registered holder or by his, her or its attorney duly authorized in writing. All rights with respect to the Preferred Stock converted pursuant to Subsection 5.1, including the rights, if any, to receive notices and vote (other than as a holder of Common Stock), will terminate at the Mandatory Conversion Time (notwithstanding the failure of the holder or holders thereof to surrender any certificates at or prior to such time), except only the rights of the holders thereof, upon surrender of any certificate or certificates of such holders (or lost certificate affidavit and agreement) therefor, to receive the items provided for in the next sentence of this Subsection 5.2. As soon as practicable after the Mandatory Conversion Time and, if applicable, the surrender of any certificate or certificates (or lost certificate affidavit and agreement) for Preferred Stock, the Corporation shall (a) issue and deliver to such holder, or to his, her or its nominees, a certificate or certificates for the number of full shares of Common Stock issuable on such conversion in accordance with the provisions hereof and (b) pay cash as provided in Subsection 4.2 in lieu of any fraction of a share of Common Stock otherwise issuable upon such conversion and the payment of any declared but unpaid dividends on the shares of Preferred Stock converted. Such converted Preferred Stock shall be retired and cancelled and may not be reissued as shares of Preferred Stock, and the Corporation may thereafter take such appropriate action (without the need for stockholder action) as may be necessary to reduce the authorized number of shares of Preferred Stock and the authorized number of shares of the applicable series of Preferred Stock accordingly.

6. Redeemed or Otherwise Acquired Shares. Any shares of Preferred Stock that are redeemed or otherwise acquired by the Corporation or any of its subsidiaries shall be automatically and immediately cancelled and retired and shall not be reissued, sold or transferred. Neither the Corporation nor any of its subsidiaries may exercise any voting or other rights granted to the holders of Preferred Stock following redemption.

7. Waiver. Any of the rights, powers, preferences and other terms of any series of Preferred Stock set forth herein may be waived on behalf of all holders of such series of Preferred Stock by the affirmative written consent or vote of the holders of at least a majority of the shares of such series of Preferred Stock then outstanding. Any of the rights, powers, preferences and other terms of the Preferred Stock as a class set forth herein may be waived on behalf of all holders of such Preferred Stock by the Requisite Holders.

8. Notices. Any notice required or permitted by the provisions of this Article Fourth to be given to a holder of shares of Preferred Stock shall be mailed, postage prepaid, to the post office address last shown on the records of the Corporation, or given by electronic communication in compliance with the provisions of the General Corporation Law, and shall be deemed sent upon such mailing or electronic transmission.

FIFTH: Subject to any additional vote required by the certificate of Incorporation or bylaws of the corporation, in furtherance and not in limitation of the powers conferred by statute, the Board of Directors is expressly authorized to make, repeal, alter, amend and rescind any or all of the bylaws of the Corporation.

SIXTH: Subject to any additional vote required by the certificate of incorporation of the Corporation, the number of directors of the Corporation shall be determined in the manner set forth in the bylaws of the Corporation.

SEVENTH: Elections of directors need not be by written ballot unless the bylaws of the Corporation shall so provide.

EIGHTH: Meetings of stockholders may be held within or without the State of Delaware, as the bylaws of the Corporation may provide. The books of the Corporation may be kept outside the State of Delaware at such place or places as may be designated from time to time by the Board of Directors or in the bylaws of the Corporation.

NINTH: To the fullest extent permitted by law, a director of the Corporation shall not be personally liable to the Corporation or its stockholders for monetary damages for breach of fiduciary duty as a director. If the General Corporation Law or any other law of the State of Delaware is amended after approval by the stockholders of this Article Ninth to authorize corporate action further eliminating or limiting the personal liability of directors, then the liability of a director of the Corporation shall be eliminated or limited to the fullest extent permitted by the General Corporation Law as so amended.

Any repeal or modification of the foregoing provisions of this Article Ninth by the stockholders of the Corporation shall not adversely affect any right or protection of a director of the Corporation existing at the time of, or increase the liability of any director of the Corporation with respect to any acts or omissions of such director occurring prior to, such repeal or modification.

TENTH: To the fullest extent permitted by applicable law, the Corporation is authorized to provide indemnification of (and advancement of expenses to) directors, officers and agents of the Corporation (and any other persons to which General Corporation Law permits the Corporation to provide indemnification) through bylaw provisions, agreements with such agents or other persons, vote of stockholders or disinterested directors or otherwise, in excess of the indemnification and advancement otherwise permitted by Section 145 of the General Corporation Law.

Any amendment, repeal or modification of the foregoing provisions of this Article Tenth shall not adversely affect any right or protection of any director, officer or other agent of the Corporation existing at the time of such amendment, repeal or modification.

ELEVENTH: The Corporation renounces, to the fullest extent permitted by law, any interest or expectancy of the Corporation in, or in being offered an opportunity to participate in, any Excluded Opportunity. An "**Excluded Opportunity**" is any matter, transaction or interest that is presented to, or acquired, created or developed by, or which otherwise comes into the possession of (i) any director of the Corporation who is not an employee of the Corporation or any of its subsidiaries, or (ii) any holder of Preferred Stock or any partner, member, director, stockholder, employee or agent of any

such holder, other than someone who is an employee of the Corporation or any of its subsidiaries (collectively, "**Covered Persons**"), unless such matter, transaction or interest is presented to, or acquired, created or developed by, or otherwise comes into the possession of, a Covered Person expressly and solely in such Covered Person's capacity as a director of the Corporation.

TWELFTH: Unless the Corporation consents in writing to the selection of an alternative forum, the Court of Chancery in the State of Delaware shall be the sole and exclusive forum for any stockholder (including a beneficial owner) to bring (i) any derivative action or proceeding brought on behalf of the Corporation, (ii) any action asserting a claim of breach of fiduciary duty owed by any director, officer or other employee of the Corporation to the Corporation or the Corporation's stockholders, (iii) any action asserting a claim against the Corporation, its directors, officers or employees arising pursuant to any provision of the Delaware General Corporation Law or the Corporation's certificate of incorporation or bylaws or (iv) any action asserting a claim against the Corporation, its directors, officers or employees governed by the internal affairs doctrine, except for, as to each of (i) through (iv) above, any claim as to which the Court of Chancery determines that there is an indispensable party not subject to the jurisdiction of the Court of Chancery (and the indispensable party does not consent to the personal jurisdiction of the Court of Chancery within 10 days following such determination), which is vested in the exclusive jurisdiction of a court or forum other than the Court of Chancery, or for which the Court of Chancery does not have subject matter jurisdiction. If any provision or provisions of this Article Twelfth shall be held to be invalid, illegal or unenforceable as applied to any person or entity or circumstance for any reason whatsoever, then, to the fullest extent permitted by law, the validity, legality and enforceability of such provisions in any other circumstance and of the remaining provisions of this Article Twelfth (including, without limitation, each portion of any sentence of this Article Twelfth containing any such provision held to be invalid, illegal or unenforceable that is not itself held to be invalid, illegal or unenforceable) and the application of such provision to other persons or entities and circumstances shall not in any way be affected or impaired thereby.

* * *

3. That the foregoing amendment and restatement was approved by the holders of the requisite number of shares of this corporation in accordance with Section 228 of the General Corporation Law.

4. That this Amended and Restated Certificate of Incorporation, which restates and integrates and further amends the provisions of this Corporation's Amended and Restated Certificate of Incorporation, has been duly adopted in accordance with Sections 242 and 245 of the General Corporation Law.

IN WITNESS WHEREOF, this Amended and Restated Certificate of Incorporation has been executed by a duly authorized officer of this corporation on this 15th day of October, 2018.

By: /s/ Edward Kaye, M.D.
Chief Executive Officer

CERTIFICATE OF AMENDMENT
TO THE
AMENDED AND RESTATED CERTIFICATE OF INCORPORATION
OF
STOKE THERAPEUTICS, INC.

Stoke Therapeutics, Inc. (the "***Corporation***"), a corporation duly organized and existing under the General Corporation Law of the State of Delaware (the "***DGCL***"), does hereby certify that the following amendment to the Corporation's Amended and Restated Certificate of Incorporation, filed with the Delaware Secretary of State on October 15, 2018 (the "***Current Certificate***"), has been duly adopted in accordance with the provisions of Section 242 of the Delaware General Corporation Law, with the approval of such amendment by the Corporation's stockholders having been given by written consent without a meeting in accordance with Sections 228(d) and 242 of the DGCL:

1. The following two paragraphs are hereby added to precede the first paragraph of Article FOURTH of the Current Certificate:

"Contingent and effective upon the filing of this Certificate of Amendment to the Amended and Restated Certificate of Incorporation (the "***Certificate of Amendment***"), every 9.94747751524931 outstanding shares of Common Stock and Preferred Stock will be combined into and automatically, without any further action by the Corporation or the stockholders thereof, become one outstanding share of Common Stock and Preferred Stock, respectively, of the Corporation (the "***Reverse Stock Split***"). No fractional share shall be issued in connection with the foregoing combination of the shares pursuant to the Reverse Split. The Corporation will pay in cash the fair value of such fractional shares, without interest and as determined in good faith by the Board of Directors of the Corporation when those entitled to receive such fractional shares are determined.

The Reverse Stock Split shall occur automatically without any further action by the holders of Common Stock or Preferred Stock, and whether or not the certificates representing such shares have been surrendered to the Corporation; *provided, however*, that the Corporation shall not be obligated to issue certificates evidencing the shares of Common Stock or Preferred Stock issuable as a result of the Reverse Stock Split unless the existing certificates evidencing the applicable shares of stock prior to the Reverse Stock Split are either delivered to the Corporation, or the holder notifies the Corporation that such certificates have been lost, stolen or destroyed, and executes an agreement satisfactory to the Corporation to indemnify the Corporation from any loss incurred by it in connection with such certificates."

2. The foregoing amendment to the Current Certificate has been duly approved by the Corporation's Board of Directors in accordance with Sections 141 and 242 of the DGCL.

3. The foregoing amendment to Current Certificate has been duly approved by the Corporation's stockholders in accordance with Sections 228 and 242 of the DGCL.

4. This Certificate of Amendment shall be effective upon filing with the Delaware Secretary of State.

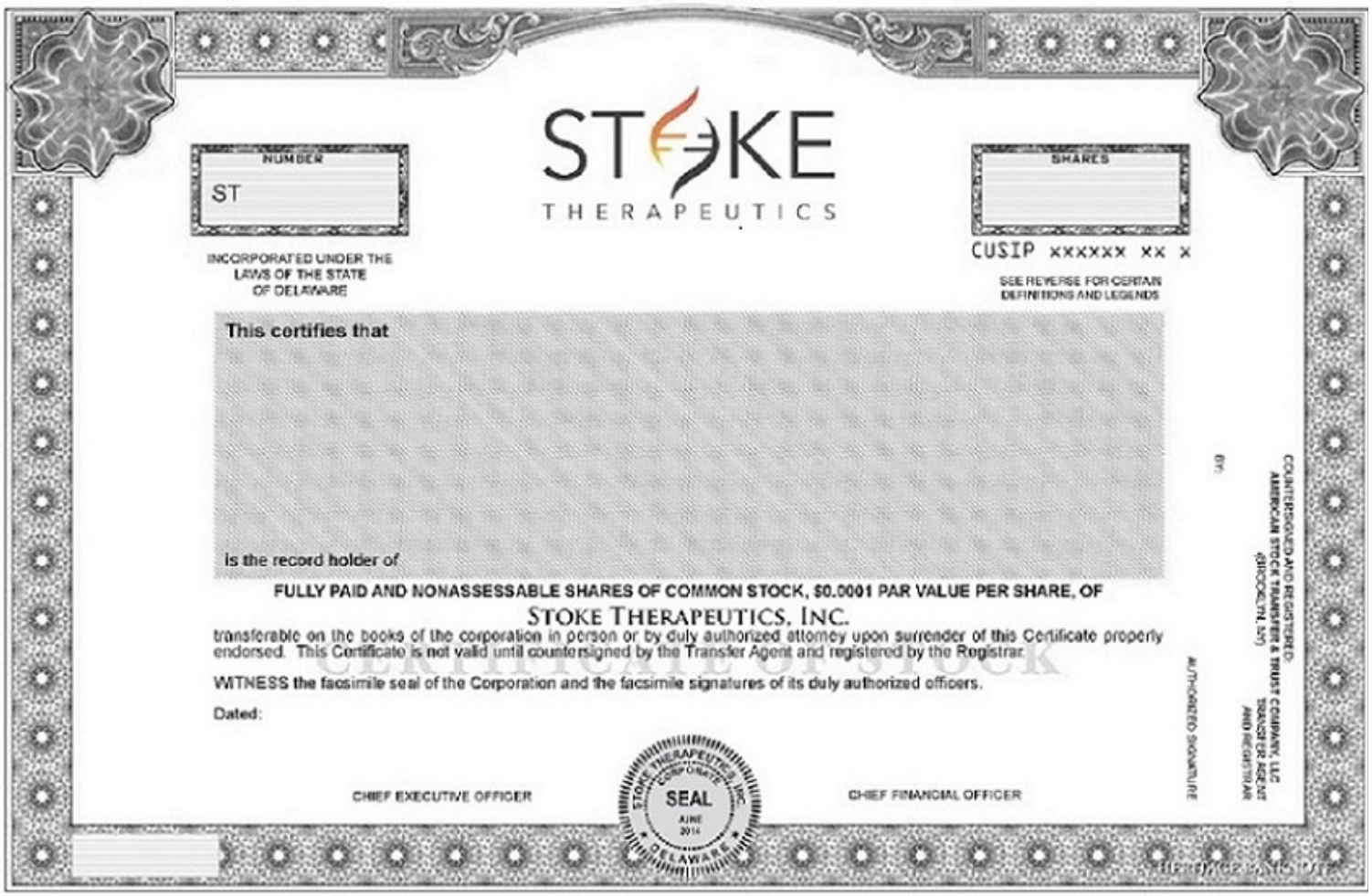
[SIGNATURE PAGE FOLLOWS]

IN WITNESS WHEREOF, the Corporation has caused this Certificate of Amendment to be signed by its duly authorized officer this 6th day of June, 2019 and the foregoing facts stated herein are true and correct.

STOKE THERAPEUTICS, INC.

By: /s/ Edward M. Kaye
Name: Edward M. Kaye, M.D.
Title: Chief Executive Officer

Exhibit 4.1


STOKE
THERAPEUTICS
NUMBER
ST
SHARES
CUSIP xxxxxx xx x
INCORPORATED UNDER THE LAWS OF THE STATE OF DELAWARE
SEE REVERSE FOR CERTAIN DEFINITIONS AND LEGENDS
This certifies that
is the record holder of
FULLY PAID AND NONASSESSABLE SHARES OF COMMON STOCK, $0.0001 PAR VALUE PER SHARE, OF
STOKE THERAPEUTICS, INC.
transferable on the books of the corporation in person or by duly authorized attorney upon surrender of this Certificate properly endorsed. This Certificate is not valid until countersigned by the Transfer Agent and registered by the Registrar.
WITNESS the facsimile seal of the Corporation and the facsimile signatures of its duly authorized officers.
Dated:
CHIEF EXECUTIVE OFFICER
SEAL
CHIEF FINANCIAL OFFICER
COUNTERSIGNED AND REGISTERED:
AMERICAN STOCK TRANSFER & TRUST COMPANY, LLC
(BROOKLYN, NY)
TRANSFER AGENT AND REGISTRAR
BY:
AUTHORIZED SIGNATURE

The Corporation shall furnish without charge to each stockholder who so requests a statement of the powers, designations, preferences and relative, participating, optional or other special rights of each class of stock of the Corporation or series thereof and the qualifications, limitations or restrictions of such preferences and/or rights. Such requests shall be made to the Corporation's Secretary at the principal office of the Corporation.

KEEP THIS CERTIFICATE IN A SAFE PLACE. IF IT IS LOST, STOLEN, OR DESTROYED THE CORPORATION WILL REQUIRE A BOND INDEMNITY AS A CONDITION TO THE ISSUANCE OF A REPLACEMENT CERTIFICATE.

The following abbreviations, when used in the inscription on the face of this certificate, shall be construed as though they were written out in full according to applicable laws or regulations:

TEN COM – as tenants in common
TEN ENT – as tenants by the entireties
JT TEN – as joint tenants with right of survivorship and not as tenants in common
COM PROP – as community property

UNIF GIFT MIN ACT – _____ Custodian _____
(Cust) (Minor)
under Uniform Gifts to Minors Act _____
(State)

UNIF TRF MIN ACT – _____ Custodian (until age _____)
(Cust)
_____ under Uniform Transfers
(Minor)
to Minors Act _____
(State)

Additional abbreviations may also be used though not in the above list.

FOR VALUE RECEIVED, _____ hereby sell(s), assign(s) and transfer(s) unto

PLEASE INSERT SOCIAL SECURITY OR OTHER IDENTIFYING NUMBER OF ASSIGNEE

_____

(PLEASE PRINT OR TYPEWRITE NAME AND ADDRESS, INCLUDING ZIP CODE, OF ASSIGNEE)

_____

_____

_____ shares of the capital stock represented by within Certificate, and do hereby irrevocably constitute and appoint

_____ attorney-in-fact to transfer the said stock on the books of the within named Corporation with full power of the substitution in the premises.

Dated _____

X _____

X _____

Signature(s) Guaranteed:

NOTICE: THE SIGNATURE TO THIS ASSIGNMENT MUST CORRESPOND WITH THE NAME AS WRITTEN UPON THE FACE OF THE CERTIFICATE IN EVERY PARTICULAR, WITHOUT ALTERATION OR ENLARGEMENT OR ANY CHANGE WHATSOEVER.

By _____

THE SIGNATURE(S) SHOULD BE GUARANTEED BY AN ELIGIBLE GUARANTOR INSTITUTION, (BANKS, STOCKBROKERS, SAVINGS AND LOAN ASSOCIATIONS AND CREDIT UNIONS WITH MEMBERSHIP IN AN APPROVED SIGNATURE GUARANTEE MEDALLION PROGRAM), PURSUANT TO S.E.C. RULE 17Ad-15. GUARANTEES BY A NOTARY PUBLIC ARE NOT ACCEPTABLE. SIGNATURE GUARANTEES MUST NOT BE DATED.

EXHIBIT 5.1

FENWICK & WEST LLP

555 CALIFORNIA STREET, 12TH FLOOR SAN FRANCISCO, CA 94104

TEL 415.875.2300 FAX 415.281.1350 WWW.FENWICK.COM

June 7, 2019

Stoke Therapeutics, Inc.
45 Wiggins Avenue
Bedford, MA 01730

Ladies and Gentlemen:

At your request, we have examined the Registration Statement on Form S-1 (File Number 333-231700) (the "***Registration Statement***") initially filed by Sutro Biopharma, Inc., a Delaware corporation (the "***Company***"), with the Securities and Exchange Commission (the "***Commission***") on or about May 23, 2019, as subsequently amended on June 7, 2019, in connection with the registration under the Securities Act of 1933, as amended ("***Securities Act***"), of an aggregate of 7,705,000 shares of the Company's Common Stock (the "***Stock***").

In connection with our opinion expressed below we have examined originals or copies of the underwriting agreement pursuant to which the Stock will be sold to the underwriters, the Registration Statement, the prospectus prepared in connection with the Registration Statement (the "***Prospectus***"), the Company's certificate of incorporation, as amended (the "***Certificate***"), and the Company's bylaws, as amended (the "***Bylaws***"), certain minutes and consents of the Company's board of directors (the "***Board***") or a committee or committees thereof and the Company's stockholders relating to the Registration Statement, the Certificate and the Bylaws, and such other agreements, documents, certificates and statements of the Company, its transfer agent and public or government officials, as we have deemed advisable, and have examined such questions of law as we have considered necessary. In giving our opinion, we have also relied upon a good standing certificate regarding the Company issued by the Delaware Secretary of State and a management certificate addressed to us and dated of even date herewith executed by the Company containing certain factual representations by the Company.

In our examination of documents for purposes of this opinion, we have assumed, and express no opinion as to, the genuineness of all signatures on original documents, the authenticity and completeness of all documents submitted to us as originals, the conformity to originals and completeness of all documents submitted to us as copies, the legal capacity of all persons or entities executing the same (other than the Company), the lack of any undisclosed termination, modification, waiver or amendment to any document reviewed by us.

We render this opinion only with respect to, and express no opinion herein concerning the application or effect of the laws of any jurisdiction other than, the existing Delaware General Corporation Law.

In connection with our opinion expressed below, we have assumed that, at or prior to the time of the delivery of any shares of Stock, the Registration Statement will have been declared effective under the Securities Act that the registration will apply to the offer and sale of such shares of Stock and will not have been modified or rescinded and that there will not have occurred any change in law affecting the validity of the issuance of such shares of Stock.

Based upon the foregoing, we are of the opinion that the up to 7,705,000 shares of Stock that may be issued and sold by the Company, when issued, sold and delivered in the manner and for the consideration stated in the Registration Statement and the Prospectus and in accordance with the resolutions adopted by the Board and to be adopted by the Pricing Committee of the Board, will be validly issued, fully paid and nonassessable.

We consent to the use of this opinion as an exhibit to the Registration Statement and further consent to all references to us, if any, in the Registration Statement, the Prospectus constituting a part thereof and any amendments thereto.

This opinion is intended solely for use in connection with issuance and sale of shares of Stock subject to the Registration Statement and is not to be relied upon for any other purpose. This opinion is rendered as of the date first written above and is based solely on our understanding of facts in existence as of such date after the aforementioned examination. In rendering the opinions above, we are opining only as to the specific legal issues expressly set forth therein, and no opinion shall be inferred as to any other matter or matters. We assume no obligation to advise you of any fact, circumstance, event or change in the law or the facts that may hereafter be brought to our attention whether or not such occurrence would affect or modify any of the opinions expressed herein.

Very truly yours,

/s/ Fenwick & West LLP

FENWICK & WEST LLP

Exhibit 10.1

INDEMNITY AGREEMENT

This Indemnity Agreement, dated as of , 2019 is made by and between Stoke Therapeutics, Inc., a Delaware corporation (the "***Company***"), and , a director, officer or key employee of the Company or one of the Company's subsidiaries or other service provider who satisfies the definition of Indemnifiable Person set forth below ("***Indemnitee***").

RECITALS

A. The Company is aware that competent and experienced persons are increasingly reluctant to serve as representatives of corporations unless they are protected by comprehensive liability insurance and indemnification, due to increased exposure to litigation costs and risks resulting from their service to such corporations, and due to the fact that the exposure frequently bears no relationship to the compensation of such representatives;

B. The members of the Board of Directors of the Company (the "***Board***") have concluded that to retain and attract talented and experienced individuals to serve as representatives of the Company and its Subsidiaries and Affiliates and to encourage such individuals to take the business risks necessary for the success of the Company and its Subsidiaries and Affiliates, it is necessary for the Company to contractually indemnify certain of its representatives and the representatives of its Subsidiaries and Affiliates, and to assume for itself maximum liability for Expenses and Other Liabilities in connection with claims against such representatives in connection with their service to the Company and its Subsidiaries and Affiliates;

C. Section 145 of the Delaware General Corporation Law ("***Section 145***"), empowers the Company to indemnify by agreement its officers, directors, employees and agents, and persons who serve, at the request of the Company, as directors, officers, employees or agents of other corporations, partnerships, joint ventures, trusts or other enterprises, and expressly provides that the indemnification provided thereby is not exclusive; and

D. The Company desires and has requested Indemnitee to serve or continue to serve as a representative of the Company and/or the Subsidiaries or Affiliates of the Company free from undue concern about inappropriate claims for damages arising out of or related to such services to the Company and/or the Subsidiaries or Affiliates of the Company.

AGREEMENT

NOW, THEREFORE, the parties hereto, intending to be legally bound, hereby agree as follows:

1. Definitions.

(a) Affiliate. For purposes of this Agreement, "***Affiliate***" of the Company means any corporation, partnership, limited liability company, joint venture, trust or other enterprise in respect of which Indemnitee is or was or will be serving as a director, officer, trustee, manager, member, partner, employee, agent, attorney, consultant, member of the entity's governing body (whether constituted as a board of directors, board of managers, general partner or otherwise), fiduciary, or in any other similar capacity at the request, election or direction of the Company, and including, but not limited to, any employee benefit plan of the Company or a Subsidiary or Affiliate of the Company.

(b) Change in Control. For purposes of this Agreement, "***Change in Control***" means (i) any "person" (as such term is used in Sections 13(d) and 14(d) of the Securities Exchange Act of 1934, as amended), other than a Subsidiary or a trustee or other fiduciary holding securities under an employee benefit plan of the Company or Subsidiary, is or becomes the "Beneficial Owner" (as defined in Rule 13d-3 under said Act), directly or indirectly,

of securities of the Company representing 50% or more of the total voting power represented by the Company's then outstanding capital stock or (ii) during any period of two consecutive years, individuals who at the beginning of such period constitute the Board and any new director whose election by the Board or nomination for election by the Company's stockholders was approved by a vote of at least two-thirds (2/3) of the directors then still in office who either were directors at the beginning of the period or whose election or nomination for election was previously so approved, cease for any reason to constitute a majority thereof, or (iii) the stockholders of the Company approve a merger or consolidation of the Company with any other corporation, other than a merger or consolidation that would result in the outstanding capital stock of the Company outstanding immediately prior thereto continuing to represent (either by remaining outstanding or by being converted into capital stock of the surviving entity) at least 80% of the total voting power represented by the capital stock of the Company or such surviving entity outstanding immediately after such merger or consolidation, or the stockholders of the Company approve a plan of complete liquidation of the Company or an agreement for the sale or disposition by the Company (in one transaction or a series of transactions) of all or substantially all of the Company's assets.

(c) Expenses. For purposes of this Agreement, "***Expenses***" means all direct and indirect costs of any type or nature whatsoever (including, without limitation, all attorneys' fees and related disbursements, and other out-of-pocket costs), paid or incurred by Indemnitee in connection with either the investigation, defense or appeal of, or being a witness in, a Proceeding (as defined below), or establishing or enforcing a right to indemnification under this Agreement, Section 145 or otherwise; provided, however, that Expenses shall not include any judgments, fines, ERISA excise taxes or penalties or amounts paid in settlement of a Proceeding.

(d) Indemnifiable Event. For purposes of this Agreement, "***Indemnifiable Event***" means any event or occurrence related to Indemnitee's service for the Company or any Subsidiary or Affiliate as an Indemnifiable Person (as defined below), or by reason of anything done or not done, or any act or omission, by Indemnitee in any such capacity.

(e) Indemnifiable Person. For the purposes of this Agreement, "***Indemnifiable Person***" means any person who is or was a director, officer, trustee, manager, member, partner, employee, attorney, consultant, member of an entity's governing body (whether constituted as a board of directors, board of managers, general partner or otherwise) or other agent or fiduciary of the Company or a Subsidiary or Affiliate of the Company.

(f) Independent Counsel. For purposes of this Agreement, "***Independent Counsel***" means legal counsel that has not performed services for the Company or Indemnitee in the five years preceding the time in question and that would not, under applicable standards of professional conduct, have a conflict of interest in representing either the Company or Indemnitee.

(g) Independent Director. For purposes of this Agreement, "***Independent Director***" means a member of the Board who is not a party to the Proceeding for which a claim is made under this Agreement.

(h) Other Liabilities. For purposes of this Agreement, "***Other Liabilities***" means any and all liabilities of any type whatsoever (including, but not limited to, judgments, fines, penalties, ERISA (or other benefit plan related) excise taxes or penalties, and amounts paid in settlement and all interest, taxes, assessments and other charges paid or payable in connection with or in respect of any such judgments, fines, ERISA (or other benefit plan related) excise taxes or penalties, or amounts paid in settlement).

(i) Proceeding. For the purposes of this Agreement, "***Proceeding***" means any threatened, pending, or completed action, suit or other proceeding, whether civil, criminal, administrative, investigative, legislative or any other type whatsoever, preliminary, informal or formal, including any arbitration or other alternative dispute resolution and including any appeal of any of the foregoing.

(j) Subsidiary. For purposes of this Agreement, "***Subsidiary***" means any entity of which more than 50% of the outstanding voting securities is owned directly or indirectly by the Company.

2. Agreement to Serve. The Indemnitee agrees to serve and/or continue to serve as an Indemnifiable Person in the capacity or capacities in which Indemnitee currently serves the Company as an Indemnifiable Person, and any additional capacity in which Indemnitee may agree to serve, until such time as Indemnitee's service in a particular capacity shall end according to the terms of an agreement, the Company's Certificate of Incorporation or Bylaws, governing law, or otherwise. Nothing contained in this Agreement is intended to create any right to continued employment or other form of service for the Company or a Subsidiary or Affiliate of the Company by Indemnitee.

3. Mandatory Indemnification.

(a) Agreement to Indemnify. In the event Indemnitee is a person who was or is a party to or witness in or is threatened to be made a party to or witness in any Proceeding by reason of an Indemnifiable Event, the Company shall indemnify Indemnitee from and against any and all Expenses and Other Liabilities incurred by Indemnitee in connection with (including in preparation for) such Proceeding to the fullest extent not prohibited by the provisions of the Company's Bylaws and the Delaware General Corporation Law ("***DGCL***"), as the same may be amended from time to time (but only to the extent that such amendment permits the Company to provide broader indemnification rights than the Bylaws or the DGCL permitted prior to the adoption of such amendment).

(b) Exception for Amounts Covered by Insurance and Other Sources. Notwithstanding the foregoing, the Company shall not be obligated to indemnify Indemnitee for Expenses or Other Liabilities of any type whatsoever (including, but not limited to judgments, fines, penalties, ERISA excise taxes or penalties and amounts paid in settlement) to the extent such have been paid directly to Indemnitee (or paid directly to a third party on Indemnitee's behalf) by any directors and officers, or other type, of insurance maintained by the Company; provided, however, that payment made to Indemnitee pursuant to an insurance policy purchased and maintained by Indemnitee at his or her own expense of any amounts otherwise indemnifiable or obligated to be made pursuant to this Agreement shall not reduce the Company's obligations to Indemnitee pursuant to this Agreement.

(c) Company Obligations Primary. The Company hereby acknowledges that Indemnitee may have rights to indemnification for Expenses and Other Liabilities provided by a venture capital firm or other sponsoring organization ("***Other Indemnitor***"). The Company agrees with Indemnitee that the Company is the indemnitor of first resort of Indemnitee with respect to matters for which indemnification is provided under this Agreement and that the Company will be obligated to make all payments due to or for the benefit of Indemnitee under this Agreement without regard to any rights that Indemnitee may have against the Other Indemnitor. The Company hereby waives any equitable rights to contribution or indemnification from the Other Indemnitor in respect of any amounts paid to indemnitee hereunder. The Company further agrees that no reimbursement of Other Liabilities or payment of Expenses by the Other Indemnitor to or for the benefit of Indemnitee shall affect the obligations of the Company hereunder, and that the Company shall be obligated to repay the Other Indemnitor for all amounts so paid or reimbursed to the extent that the Company has an obligation to indemnify Indemnitee for such Expenses or Other Liabilities hereunder.

4. Partial Indemnification. If Indemnitee is entitled under any provision of this Agreement to indemnification by the Company for some or a portion of any Expenses or Other Liabilities but not entitled, however, to indemnification for the total amount of such Expenses or Other Liabilities, the Company shall nevertheless indemnify Indemnitee for such total amount except as to the portion thereof for which indemnification is prohibited by the provisions of the Company's Bylaws or the DGCL. In any review or Proceeding to determine the extent of indemnification, the Company shall bear the burden to establish, by clear and convincing evidence, the lack of a successful resolution of a particular claim, issue or matter and which amounts sought in indemnity are allocable to claims, issues or matters which were not successfully resolved.

5. Liability Insurance. So long as Indemnitee shall continue to serve the Company or a Subsidiary or Affiliate of the Company as an Indemnifiable Person and thereafter so long as Indemnitee shall be subject to any possible claim or threatened, pending or completed Proceeding as a result of an Indemnifiable Event, the Company shall use reasonable efforts to maintain in full force and effect for the benefit of Indemnitee as an insured (i) liability insurance issued by one or more reputable insurers and having the policy amount and deductible deemed appropriate by the Board and providing in all respects coverage at least comparable to and in the same amount as that provided to the Chairman of the Board or the Chief Executive Officer of the Company and (ii) any replacement or substitute policies issued by one or more reputable insurers providing in all respects coverage at least comparable to and in the same amount as that being provided to the Chairman of the Board or the Chief Executive Officer of the Company. The purchase, establishment and maintenance of any such insurance or other arrangements shall not in any way limit or affect the rights and obligations of the Company or of Indemnitee under this Agreement except as expressly provided herein, and the execution and delivery of this Agreement by the Company and Indemnitee shall not in any way limit or affect the rights and obligations of the Company or the other party or parties thereto under any such insurance or other arrangement. In the event of a Change in Control subsequent to the date of this Agreement, or the Company's becoming insolvent, including being placed into receivership or entering the federal bankruptcy process,

the Company shall maintain in force any and all insurance policies then maintained by the Company in providing insurance—directors' and officers' liability, fiduciary, employment practices or otherwise—in respect of the individual directors and officers of the Company, for a fixed period of six years thereafter. Such coverage shall be non-cancelable and shall be placed and serviced by the Company's incumbent insurance broker or a broker selected by a majority of the Independent Directors.

6. Mandatory Advancement of Expenses. If requested by Indemnitee, the Company shall advance prior to the final disposition of the Proceeding all Expenses reasonably incurred by Indemnitee in connection with (including in preparation for) a Proceeding related to an Indemnifiable Event within (30) days after the receipt by the Company of a statement or statements from Indemnitee requesting such advance or advances from time to time, whether prior to or after final disposition of such Proceeding. Such statement or statements shall reasonably evidence the Expenses incurred by Indemnitee. The right to advances under this section shall in all events continue until final disposition of any Proceeding, including any appeal therein. Indemnitee hereby undertakes to repay such amounts advanced if, and only if and to the extent that, it shall ultimately be determined that Indemnitee is not entitled to be indemnified by the Company under the provisions of this Agreement, the Company's Bylaws or the DGCL, and no additional form of undertaking with respect to such obligation to repay shall be required. Indemnitee's undertaking to repay any Expenses advanced to Indemnitee hereunder shall be unsecured and shall not be subject to the accrual or payment of any interest thereon. In the event that Indemnitee's request for the advancement of expenses shall be accompanied by an affidavit of counsel to Indemnitee to the effect that such counsel has reviewed such Expenses and that such Expenses are reasonable in such counsel's view, then such expenses shall be deemed reasonable in the absence of clear and convincing evidence to the contrary.

7. Notice and Other Indemnification Procedures.

(a) Notification. Promptly after receipt by Indemnitee of notice of the commencement of or the threat of commencement of any Proceeding, unless the Company is a named co-defendant with Indemnitee, Indemnitee shall, if Indemnitee believes that indemnification or advancement of Expenses with respect thereto may be sought from the Company under this Agreement, notify the Company of the commencement or threat of commencement thereof. However, a failure so to notify the Company promptly following Indemnitee's receipt of such notice shall not relieve the Company from any liability that it may have to Indemnitee except to the extent that the Company is materially prejudiced in its defense of such Proceeding as a result of such failure.

(b) Insurance and Other Matters. If, at the time of the receipt of a notice of the commencement of a Proceeding pursuant to Section 7(a) above, the Company has director and officer liability insurance in effect, the Company shall give prompt notice of the commencement of such Proceeding to the issuers in accordance with the procedures set forth in the respective policies. The Company shall thereafter take all reasonable action to cause such insurers to pay, on behalf of Indemnitee, all amounts payable as a result of such Proceeding in accordance with the terms of such insurance policies. In addition, the Company will instruct the insurers and the Company's insurance broker that they may communicate directly with Indemnitee regarding such claim.

(c) Assumption of Defense. In the event the Company shall be obligated to advance the Expenses for any Proceeding against Indemnitee, the Company, if deemed appropriate by the Company, shall be entitled to assume the defense of such Proceeding as provided herein. Such defense by the Company may include the representation of two or more parties by one attorney or law firm as permitted under the ethical rules and legal requirements related to joint representations. Following delivery of written notice to Indemnitee of the Company's election to assume the defense of such Proceeding, the approval by Indemnitee (which approval shall not be unreasonably withheld) of counsel designated by the Company and the retention of such counsel by the Company, the Company will not be liable to Indemnitee under this Agreement for any fees and expenses of counsel subsequently incurred by Indemnitee with respect to the same Proceeding. If (A) the employment of counsel by Indemnitee has been previously authorized by the Company, (B) Indemnitee shall have notified the Board in writing that Indemnitee has reasonably concluded that there may be a conflict of interest between the Company and Indemnitee in the conduct of any such defense, (C) the Company fails to employ counsel to assume the defense of such Proceeding, or (D) after a Change in Control, the employment of counsel by Indemnitee has been approved by the Independent Counsel, the Expenses related to work conducted by Indemnitee's counsel shall be subject to indemnification and/or advancement pursuant to the terms of this Agreement. Nothing herein shall prevent Indemnitee from employing counsel for any such Proceeding at Indemnitee's expense. Indemnitee agrees that any such separate counsel retained by Indemnitee will be a member of any approved list of panel counsel under the Company's applicable directors' and officers' insurance policy, should the applicable policy provide for a panel of approved counsel.

(d) Settlement. The Company shall not be liable to indemnify Indemnitee under this Agreement or otherwise for any amounts paid in settlement of any Proceeding effected without the Company's written consent; provided, however, that if a Change in Control has occurred subsequent to the date of this Agreement, the Company shall be liable for indemnification of Indemnitee for amounts paid in settlement if the Independent Counsel has approved the settlement. Neither the Company nor any Subsidiary or Affiliate shall enter into a settlement of any Proceeding that might result in the imposition of any Expense, Other Liability, penalty, limitation or detriment on Indemnitee, whether indemnifiable under this Agreement or otherwise, without Indemnitee's written consent. Neither the Company nor Indemnitee shall unreasonably withhold consent from any settlement of any Proceeding. The Company shall promptly notify Indemnitee upon the Company's receipt of an offer to settle, or if the Company makes an offer to settle, any Proceeding, and provide Indemnitee with a reasonable amount of time to consider such settlement, in the case of any such settlement for which the consent of Indemnitee would be required hereunder. The Company shall not, on its own behalf, settle any part of any Proceeding to which Indemnitee is a party with respect to other parties (including the Company) without the written consent of Indemnitee if any portion of the settlement is to be funded from insurance proceeds unless approved by a majority of the Independent Directors, provided that this sentence shall cease to be of any force and effect if it has been determined in accordance with this Agreement that Indemnitee is not entitled to indemnification hereunder with respect to such Proceeding or if the Company's obligations hereunder to Indemnitee with respect to such Proceeding have been fully discharged.

8. Determination of Right to Indemnification.

(a) Success on the Merits or Otherwise. To the extent that Indemnitee has been successful on the merits or otherwise in defense of any Proceeding referred to in Section 3(a) above or in the defense of any claim, issue or matter described therein, the Company shall indemnify Indemnitee against Expenses actually and reasonably incurred in connection therewith.

(b) Indemnification in Other Situations. In the event that Section 8(a) is inapplicable, the Company shall also indemnify Indemnitee if Indemnitee has not failed to meet the applicable standard of conduct for indemnification.

(c) Forum. Indemnitee shall be entitled to select the forum in which determination of whether or not Indemnitee has met the applicable standard of conduct shall be decided, and such election will be made from among the following:

a. Those members of the Board who are Independent Directors even though less than a quorum;

b. A committee of Independent Directors designated by a majority vote of Independent Directors, even though less than a quorum; or

c. Independent Counsel selected by Indemnitee and approved by the Board, which approval may not be unreasonably withheld, which counsel shall make such determination in a written opinion.

If Indemnitee is an officer or a director of the Company at the time that Indemnitee is selecting the forum, then Indemnitee shall not select Independent Counsel as such forum unless there are no Independent Directors or unless the Independent Directors agree to the selection of Independent Counsel as the forum.

The selected forum shall be referred to herein as the "Reviewing Party". Notwithstanding the foregoing, following any Change in Control subsequent to the date of this Agreement, the Reviewing Party shall be Independent Counsel selected in the manner provided in c. above.

(d) Decision Timing and Expenses. As soon as practicable, and in no event later than thirty (30) days after receipt by the Company of written notice of Indemnitee's choice of forum pursuant to Section 8(c) above, the Company and Indemnitee shall each submit to the Reviewing Party such information as they believe is appropriate for the Reviewing Party to consider. The Reviewing Party shall arrive at its decision within a reasonable period of time following the receipt of all such information from the Company and Indemnitee, but in no event later than thirty (30) days following the receipt of all such information, provided that the time by which the Reviewing Party must reach a decision may be extended by mutual agreement of the Company and Indemnitee. All Expenses associated with the process set forth in this Section 8(d), including but not limited to the Expenses of the Reviewing Party, shall be paid by the Company.

(e) Delaware Court of Chancery. Notwithstanding a final determination by any Reviewing Party that Indemnitee is not entitled to indemnification with respect to a specific Proceeding, Indemnitee shall have the right to apply to the Court of Chancery, for the purpose of enforcing Indemnitee's right to indemnification pursuant to this Agreement.

(f) Expenses. The Company shall indemnify Indemnitee against all Expenses incurred by Indemnitee in connection with any hearing or Proceeding under this Section 8 involving Indemnitee and against all Expenses and Other Liabilities incurred by Indemnitee in connection with any other Proceeding between the Company and Indemnitee involving the interpretation or enforcement of the rights of Indemnitee under this Agreement unless a court of competent jurisdiction finds that each of the material claims of Indemnitee in any such Proceeding was frivolous or made in bad faith.

(g) Determination of "Good Faith". For purposes of any determination of whether Indemnitee acted in "good faith" or acted in "bad faith," Indemnitee shall be deemed to have acted in good faith or not acted in bad faith if in taking or failing to take the action in question Indemnitee relied on the records or books of account of the Company or a Subsidiary or Affiliate, including financial statements, or on information, opinions, reports or statements provided to Indemnitee by the officers or other employees of the Company or a Subsidiary or Affiliate in the course of their duties, or on the advice of legal counsel for the Company or a Subsidiary or Affiliate, or on information or records given or reports made to the Company or a Subsidiary or Affiliate by an independent certified public accountant or by an appraiser or other expert selected by the Company or a Subsidiary or Affiliate, or by any other person (including legal counsel, accountants and financial advisors) as to matters Indemnitee reasonably believes are within such other person's professional or expert competence and who has been selected with reasonable care by or on behalf of the Company or a Subsidiary or Affiliate. In connection with any determination as to whether Indemnitee is entitled to be indemnified hereunder, or to advancement of Expenses, the Reviewing Party or court shall presume that Indemnitee has satisfied the applicable standard of conduct and is entitled to indemnification or advancement of Expenses, as the case may be, and the burden of proof shall be on the Company to establish, by clear and convincing evidence, that Indemnitee is not so entitled. The provisions of this Section 8(g) shall not be deemed to be exclusive or to limit in any way the other circumstances in which Indemnitee may be deemed to have met the applicable standard of conduct set forth in this Agreement. In addition, the knowledge and/or actions, or failures to act, of any other person serving the Company or a Subsidiary or Affiliate as an Indemnifiable Person shall not be imputed to Indemnitee for purposes of determining the right to indemnification hereunder.

9. Exceptions. Any other provision herein to the contrary notwithstanding,

(a) Claims Initiated by Indemnitee. The Company shall not be obligated pursuant to the terms of this Agreement to indemnify or advance Expenses to Indemnitee with respect to Proceedings or claims initiated or brought voluntarily by Indemnitee and not by way of defense, except (1) with respect to Proceedings brought to establish or enforce a right to indemnification under this Agreement, any other statute or law, as permitted under Section 145, or otherwise, (2) where the Board has consented to the initiation of such Proceeding, or (3) with respect to Proceedings brought to discharge Indemnitee's fiduciary responsibilities, whether under ERISA or otherwise, but such indemnification or advancement of Expenses may be provided by the Company in specific cases if the Board finds it to be appropriate; or

(b) Actions Based on Federal Statutes Regarding Profit Recovery and Return of Bonus Payments. The Company shall not be obligated pursuant to the terms of this Agreement to indemnify Indemnitee on account of (i) any suit in which judgment is rendered against Indemnitee for an accounting of profits made from the purchase or sale by Indemnitee of securities of the Company pursuant to the provisions of Section 16(b) of the Securities Exchange Act of l934 and amendments thereto or similar provisions of any federal, state or local statutory law, or (ii) any reimbursement of the Company by the Indemnitee of any bonus or other incentive-based or equity-based compensation or of any profits realized by the Indemnitee from the sale of securities of the Company, as required in each case under the Exchange Act (including any such reimbursements that arise from an accounting restatement of the Company pursuant to Section 304 of the Sarbanes-Oxley Act of 2002 (the "***Sarbanes-Oxley Act***"), or the payment to the Company of profits arising from the purchase and sale by Indemnitee of securities in violation of Section 306 of the Sarbanes-Oxley Act); or

(c) Unlawful Indemnification. The Company shall not be obligated pursuant to the terms of this Agreement to indemnify Indemnitee for Other Liabilities if such indemnification is prohibited by law as determined by a court of competent jurisdiction in a final adjudication not subject to further appeal.

10. Non-exclusivity. The provisions for indemnification and advancement of Expenses set forth in this Agreement shall not be deemed exclusive of any other rights which Indemnitee may have under any provision of law, the Company's Certificate of Incorporation or Bylaws, the vote of the Company's stockholders or disinterested directors, other agreements, or otherwise, both as to acts or omissions in his or her official capacity and to acts or omissions in another capacity while serving the Company or a Subsidiary or Affiliate as an Indemnifiable Person and Indemnitee's rights hereunder shall continue after Indemnitee has ceased serving the Company or a Subsidiary or Affiliate as an Indemnifiable Person and shall inure to the benefit of the heirs, executors and administrators of Indemnitee.

11. Severability. If any provision or provisions of this Agreement shall be held to be invalid, illegal or unenforceable for any reason whatsoever, (i) the validity, legality and enforceability of the remaining provisions of the Agreement (including, without limitation, all portions of any paragraphs of this Agreement containing any such provision held to be invalid, illegal or unenforceable, that are not themselves invalid, illegal or unenforceable) shall not in any way be affected or impaired thereby, and (ii) to the fullest extent possible, the provisions of this Agreement (including, without limitation, all portions of any paragraphs of this Agreement containing any such provision held to be invalid, illegal or unenforceable, that are not themselves invalid, illegal or unenforceable) shall be construed so as to give effect to the intent manifested by the provision held invalid, illegal or unenforceable.

12. Supersession, Modification and Waiver. This Agreement supersedes any prior indemnification agreement between the Indemnitee and the Company, its Subsidiaries or its Affiliates. If the Company and Indemnitee have previously entered into an indemnification agreement providing for the indemnification of Indemnitee by the Company, parties entry into this Agreement shall be deemed to amend and restate such prior agreement to read in its entirety as, and be superseded by, this Agreement. No supplement, modification or amendment of this Agreement shall be binding unless executed in writing by both of the parties hereto. No waiver of any of the provisions of this Agreement shall be deemed or shall constitute a waiver of any other provision hereof (whether or not similar) and except as expressly provided herein, no such waiver shall constitute a continuing waiver.

13. Successors and Assigns. The terms of this Agreement shall bind, and shall inure to the benefit of, and be enforceable by the parties hereto and their respective successors (including any direct or indirect successor by purchase, merger, consolidation or otherwise to all or substantially all of the business and/or assets of the Company), assigns, spouses, heirs and personal and legal representatives. In addition, the Company shall require and cause any successor (whether direct or indirect by purchase, merger, consolidation or otherwise) to all, substantially all, or a substantial part, of the business and/or assets of the Company, by written agreement in form and substance satisfactory to Indemnitee, expressly to assume and agree to perform this Agreement and indemnify Indemnitee to the fullest extent permitted by law.

14. Notice. All notices, requests, demands and other communications under this Agreement shall be in writing and shall be deemed duly given (i) if delivered by hand and a receipt is provided by the party to whom such communication is delivered, (ii) if mailed by certified or registered mail with postage prepaid, return receipt requested, on the signing by the recipient of an acknowledgement of receipt form accompanying delivery through the U.S. mail, (iii) by personal service by a process server, or (iv) by delivery to the recipient's address by overnight delivery (e.g., FedEx, UPS or DHL) or other commercial delivery service. Addresses for notice to either party are as shown on the signature page of this Agreement, or as subsequently modified by written notice complying with the provisions of this Section 14. Delivery of communications to the Company with respect to this Agreement shall be sent to the attention of the Company's Chief Financial Officer.

15. No Presumptions. For purposes of this Agreement, the termination of any Proceeding, by judgment, order, settlement (whether with or without court approval) or conviction, or upon a plea of nolo contendere or its

equivalent, shall not, of itself, create a presumption that Indemnitee did not meet any particular standard of conduct or have any particular belief or that a court has determined that indemnification is not permitted by applicable law or otherwise. In addition, neither the failure of the Company or a Reviewing Party to have made a determination as to whether Indemnitee has met any particular standard of conduct or had any particular belief, nor an actual determination by the Company or a Reviewing Party that Indemnitee has not met such standard of conduct or did not have such belief, prior to the commencement of Proceedings by Indemnitee to secure a judicial determination by exercising Indemnitee's rights under Section 8(e) of this Agreement shall be a defense to Indemnitee's claim or create a presumption that Indemnitee has failed to meet any particular standard of conduct or did not have any particular belief or is not entitled to indemnification under applicable law or otherwise.

16. Survival of Rights. The rights conferred on Indemnitee by this Agreement shall continue after Indemnitee has ceased to serve the Company or a Subsidiary or Affiliate of the Company as an Indemnifiable Person and shall inure to the benefit of Indemnitee's heirs, executors and administrators.

17. Subrogation and Contribution.

(a) Except as otherwise expressly provided in this Agreement, in the event of payment under this Agreement, the Company shall be subrogated to the extent of such payment to all of the rights of recovery of Indemnitee, who shall execute all documents required and shall do all acts that may be necessary to secure such rights and to enable the Company effectively to bring suit to enforce such rights.

(b) To the fullest extent permissible under applicable law, if the indemnification provided for in this Agreement is unavailable to Indemnitee for any reason whatsoever, the Company, in lieu of indemnifying Indemnitee, shall contribute to the amount incurred by or on behalf of Indemnitee, whether for judgments, fines, penalties, excise taxes, amounts paid or to be paid in settlement and/or for Expenses, in connection with any claim relating to an indemnifiable event under this Agreement, in such proportion as is deemed fair and reasonable in light of all of the circumstances of such Proceeding in order to reflect (i) the relative benefits received by the Company and Indemnitee as a result of the event(s) and/or transaction(s) giving cause to such Proceeding; and/or (ii) the relative fault of the Company (and its directors, officers, employees and agents) and Indemnitee in connection with such event(s) and/or transaction(s).

18. Specific Performance, Etc. The parties recognize that if any provision of this Agreement is violated by the Company, Indemnitee may be without an adequate remedy at law. Accordingly, in the event of any such violation, Indemnitee shall be entitled, if Indemnitee so elects, to institute Proceedings, either in law or at equity, to obtain damages, to enforce specific performance, to enjoin such violation, or to obtain any relief or any combination of the foregoing as Indemnitee may elect to pursue.

19. Counterparts. This Agreement may be executed in counterparts, each of which shall for all purposes be deemed to be an original but all of which together shall constitute one and the same agreement. Only one such counterpart signed by the party against whom enforceability is sought needs to be produced to evidence the existence of this Agreement.

20. Headings. The headings of the sections and paragraphs of this Agreement are inserted for convenience only and shall not be deemed to constitute part of this Agreement or to affect the construction or interpretation thereof.

21. Governing Law. This Agreement shall be governed exclusively by and construed according to the laws of the State of Delaware, as applied to contracts between Delaware residents entered into and to be performed entirely with Delaware.

22. Consent to Jurisdiction. The Company and Indemnitee each hereby irrevocably consent to the jurisdiction of the courts of the State of Delaware for all purposes in connection with any Proceeding which arises out of or relates to this Agreement.

[*Signature Page Follows*]

The parties hereto have entered into this Indemnity Agreement effective as of the date first above written.

STOKE THERAPEUTICS, INC.:

By: _____

Its: _____

INDEMNITEE:

_____

Address: _____

_____

STOKE THERAPEUTICS, INC.

2019 EQUITY INCENTIVE PLAN

1. PURPOSE. The purpose of this Plan is to provide incentives to attract, retain, and motivate eligible persons whose present and potential contributions are important to the success of the Company, and any Parents, Subsidiaries, and Affiliates that exist now or in the future, by offering them an opportunity to participate in the Company's future performance through the grant of Awards. Capitalized terms not defined elsewhere in the text are defined in Section 29.

2. SHARES SUBJECT TO THE PLAN.

(a) Number of Shares Available. Subject to Sections 2.6 and 22 and any other applicable provisions hereof, the total number of Shares reserved and available for grant and issuance pursuant to this Plan as of the date of adoption of the Plan by the Board, is Two Million Two Hundred Thousand (2,200,000) Shares, plus (a) any reserved Shares not issued or subject to outstanding awards granted under the Company's 2014 Equity Incentive Plan, as amended (the "***Prior Plan***") on the Effective Date (as defined below), (b) Shares that are subject to awards granted under the Prior Plan that cease to be subject to such awards by forfeiture or otherwise after the Effective Date, (c) Shares issued under the Prior Plan before or after the Effective Date pursuant to the exercise of stock options that are, after the Effective Date, forfeited, (d) Shares issued under the Prior Plan that are repurchased by the Company at the original issue price, and (e) Shares that are subject to stock options or other awards under the Prior Plan that are used to pay the exercise price of an stock option or withheld to satisfy the tax withholding obligations related to any award.

(b) Lapsed, Returned Awards. Shares subject to Awards, and Shares issued under the Plan under any Award, will again be available for grant and issuance in connection with subsequent Awards under this Plan to the extent such Shares: (a) are subject to issuance upon exercise of an Option or SAR granted under this Plan but which cease to be subject to the Option or SAR for any reason other than exercise of the Option or SAR, (b) are subject to Awards granted under this Plan that are forfeited or are repurchased by the Company at the original issue price, (c) are subject to Awards granted under this Plan that otherwise terminate without such Shares being issued or (d) are surrendered pursuant to an Exchange Program. To the extent an Award under the Plan is paid out in cash rather than Shares, such cash payment will not result in reducing the number of Shares available for issuance under the Plan. Shares used to pay the exercise price of an Award or withheld to satisfy the tax withholding obligations related to an Award will become available for grant and issuance in connection with subsequent Awards under this Plan. For the avoidance of doubt, Shares that otherwise become available for grant and issuance because of the provisions of this Section 2.2 will not include Shares subject to Awards that initially became available because of the substitution clause in Section 22.2 hereof.

(c) Minimum Share Reserve. At all times the Company will reserve and keep available a sufficient number of Shares as will be required to satisfy the requirements of all outstanding Awards granted under this Plan.

(d) Automatic Share Reserve Increase. The number of Shares available for grant and issuance under the Plan will be increased on January 1 of each of the first ten (10) calendar years during the term of the Plan by the lesser of (a) four percent (4%) of the number of shares of all classes of the Company's common stock issued and outstanding on each December 31 immediately prior to the date of increase or (b) such number of Shares determined by the Board.

(e) ISO Limitation. No more than Twenty-Two Million (22,000,000) Shares will be issued pursuant to the exercise of ISOs granted under the Plan.

(f) Adjustment of Shares. If the number of outstanding Shares is changed by a stock dividend, extraordinary dividend or distribution (whether in cash, shares, or other property, other than a regular cash dividend), recapitalization, stock split, reverse stock split, subdivision, combination, consolidation, reclassification, spin-off, or similar change in the capital structure of the Company, without consideration, then (a) the number and class of Shares reserved for issuance and future grant under the Plan set forth in Section 2.1, including Shares reserved under sub-clauses (a)-(e) of Section 2.1, (b) the Exercise Prices of and number and class of Shares subject to outstanding Options and SARs, (c) the number and class of Shares subject to other outstanding Awards and (d) the maximum number and class of Shares that may be issued as ISOs set forth in Section 2.5, will be proportionately adjusted, subject to any required action by the Board or the stockholders of the Company and in compliance with applicable securities laws, provided that fractions of a Share will not be issued.

If, by reason of an adjustment pursuant to this Section 2.6, a Participant's Award Agreement or other agreement related to any Award, or the Shares subject to such Award, covers additional or different shares of stock or securities, then such additional or different shares, and the Award Agreement or such other agreement in respect thereof, will be subject to all of the terms, conditions, and restrictions which were applicable to the Award or the Shares subject to such Award prior to such adjustment.

3. ELIGIBILITY. ISOs may be granted only to Employees. All other Awards may be granted to Employees, Consultants, Directors, and Non-Employee Directors, provided that such Consultants, Directors, and Non-Employee Directors render bona fide services not in connection with the offer and sale of securities in a capital-raising transaction.

4. ADMINISTRATION.

(a) Committee Composition; Authority. This Plan will be administered by the Committee or by the Board acting as the Committee. Subject to the general purposes, terms, and conditions of this Plan, and to the direction of the Board, the Committee will have full power to implement and carry out this Plan, except, however, the Board will establish the terms for the grant of an Award to Non-Employee Directors. The Committee will have the authority to:

(i) construe and interpret this Plan, any Award Agreement, and any other agreement or document executed pursuant to this Plan;

(ii) prescribe, amend, and rescind rules and regulations relating to this Plan or any Award;

(iii) select persons to receive Awards;

(iv) determine the form and terms and conditions, not inconsistent with the terms of the Plan, of any Award granted hereunder. Such terms and conditions include, but are not limited to, the Exercise Price, the time or times when Awards may vest and be exercised (which may be based on performance criteria) or settled, any vesting acceleration or waiver of forfeiture restrictions, the method to satisfy tax withholding obligations or any other tax liability legally due, and any restriction or limitation regarding any Award or the Shares relating thereto, based in each case on such factors as the Committee will determine;

(v) determine the number of Shares or other consideration subject to Awards;

(vi) determine the Fair Market Value in good faith and interpret the applicable provisions of this Plan and the definition of Fair Market Value in connection with circumstances that impact the Fair Market Value, if necessary;

(vii) determine whether Awards will be granted singly, in combination with, in tandem with, in replacement of, or as alternatives to, other Awards under this Plan or any other incentive or compensation plan of the Company or any Parent, Subsidiary, or Affiliate;

(viii) grant waivers of Plan or Award conditions;

(ix) determine the vesting, exercisability, and payment of Awards;

(x) correct any defect, supply any omission or reconcile any inconsistency in this Plan, any Award or any Award Agreement;

(xi) determine whether an Award has been vested and/or earned;

(xii) determine the terms and conditions of any, and to institute any Exchange Program;

(xiii) reduce or modify any criteria with respect to Performance Factors;

(xiv) adjust Performance Factors to take into account changes in law and accounting or tax rules as the Committee deems necessary or appropriate to reflect the impact of extraordinary or unusual items, events, or circumstances to avoid windfalls or hardships;

(xv) adopt terms and conditions, rules, and/or procedures (including the adoption of any subplan under this Plan) relating to the operation and administration of the Plan to accommodate requirements of local law and procedures outside of the United States or to qualify Awards for special tax treatment under laws of jurisdictions other than the United States;

(xvi) exercise discretion with respect to Performance Awards;

(xvii) make all other determinations necessary or advisable for the administration of this Plan; and

(xviii) delegate any of the foregoing to a subcommittee or to one or more executive officers pursuant to a specific delegation as permitted by applicable law, including Section 157(c) of the Delaware General Corporation Law.

(b) Committee Interpretation and Discretion. Any determination made by the Committee with respect to any Award will be made in its sole discretion at the time of grant of the Award or, unless in contravention of any express term of the Plan or Award, at any later time, and such determination will be final and binding on the Company and all persons having an interest in any Award under the Plan. Any dispute regarding the interpretation of the Plan or any Award Agreement will be submitted by the Participant or Company to the Committee for review. The resolution of such a dispute by the Committee will be final and binding on the Company and the Participant. The Committee may delegate to one or more executive officers the authority to review and resolve disputes with respect to Awards held by Participants who are not Insiders, and such resolution will be final and binding on the Company and the Participant.

(c) Section 16 of the Exchange Act. Awards granted to Participants who are subject to Section 16 of the Exchange Act must be approved by two or more "non-employee directors" (as defined in the regulations promulgated under Section 16 of the Exchange Act).

(d) Documentation. The Award Agreement for a given Award, the Plan, and any other documents may be delivered to, and accepted by, a Participant or any other person in any manner (including electronic distribution or posting) that meets applicable legal requirements.

(e) Foreign Award Recipients. Notwithstanding any provision of the Plan to the contrary, in order to comply with the laws and practices in other countries in which the Company, its Subsidiaries, and Affiliates operate or have Employees or other individuals eligible for Awards, the Committee, in its sole discretion, will have the power and authority to: (a) determine which Subsidiaries and Affiliates will be covered by the Plan; (b) determine which individuals outside the United States are eligible to participate in the Plan, which may include individuals who provide services to the Company, Subsidiary or Affiliate under an agreement with a foreign nation or agency; (c) modify the terms and conditions of any Award granted to individuals outside the United States or foreign nationals to comply with applicable foreign laws, policies, customs, and practices; (d) establish subplans and modify exercise procedures, vesting conditions, and other terms and procedures to the extent the Committee determines such actions to be necessary or advisable (and such subplans and/or modifications will be attached to this Plan as appendices, if necessary); and (e) take any action, before or after an Award is made, that the Committee determines to be necessary or advisable to obtain approval or comply with any local governmental regulatory exemptions or approvals, provided, however, that no action taken under this Section 4.5 will increase the Share limitations contained in Section 2.1 hereof. Notwithstanding the foregoing, the Committee may not take any actions hereunder, and no Awards will be granted, that would violate the Exchange Act or any other applicable United States securities law, the Code, or any other applicable United States governing statute or law.

5. OPTIONS. An Option is the right but not the obligation to purchase a Share, subject to certain conditions, if applicable. The Committee may grant Options to eligible Employees, Consultants, and Directors and will determine whether such Options will be Incentive Stock Options within the meaning of the Code ("***ISOs***") or Nonqualified Stock Options ("***NSOs***"), the number of Shares subject to the Option, the Exercise Price of the Option, the period during which the Option may vest and be exercised, and all other terms and conditions of the Option, subject to the following terms of this section.

(a) Option Grant. Each Option granted under this Plan will identify the Option as an ISO or an NSO. An Option may be, but need not be, awarded upon satisfaction of such Performance Factors during any Performance Period as are set out in advance in the Participant's individual Award Agreement. If the Option is being earned upon the satisfaction of Performance Factors, then the Committee will: (a) determine the nature, length, and starting date of any Performance Period for each Option; and (b) select from among the Performance Factors to be used to measure the performance, if any. Performance Periods may overlap and Participants may participate simultaneously with respect to Options that are subject to different performance goals and other criteria.

(b) Date of Grant. The date of grant of an Option will be the date on which the Committee makes the determination to grant such Option, or a specified future date. The Award Agreement and a copy of this Plan will be delivered to the Participant within a reasonable time after the granting of the Option.

(c) Exercise Period. Options may be vested and exercisable within the times or upon the conditions as set forth in the Award Agreement governing such Option, provided, however, that no Option will be exercisable after the expiration of ten (10) years from the date the Option is granted and

provided further that no ISO granted to a person who, at the time the ISO is granted, directly or by attribution owns more than ten percent (10%) of the total combined voting power of all classes of stock of the Company or of any Parent or Subsidiary ("***Ten Percent Stockholder***") will be exercisable after the expiration of five (5) years from the date the ISO is granted. The Committee also may provide for Options to become exercisable at one time or from time to time, periodically or otherwise, in such number of Shares or percentage of Shares as the Committee determines.

(d) Exercise Price. The Exercise Price of an Option will be determined by the Committee when the Option is granted, provided that: (a) the Exercise Price of an Option will be not less than one hundred percent (100%) of the Fair Market Value of the Shares on the date of grant, and (b) the Exercise Price of any ISO granted to a Ten Percent Stockholder will not be less than one hundred ten percent (110%) of the Fair Market Value of the Shares on the date of grant. Payment for the Shares purchased may be made in accordance with Section 12 and the Award Agreement and in accordance with any procedures established by the Company.

(e) Method of Exercise. Any Option granted hereunder will be vested and exercisable according to the terms of the Plan and at such times and under such conditions as determined by the Committee and set forth in the Award Agreement. An Option may not be exercised for a fraction of a Share. An Option will be deemed exercised when the Company receives: (a) notice of exercise (in such form as the Committee may specify from time to time) from the person entitled to exercise the Option (and/or via electronic execution through the authorized third-party administrator), and (b) full payment for the Shares with respect to which the Option is exercised (together with applicable withholding taxes). Full payment may consist of any consideration and method of payment authorized by the Committee and permitted by the Award Agreement and the Plan. Shares issued upon exercise of an Option will be issued in the name of the Participant. Until the Shares are issued (as evidenced by the appropriate entry on the books of the Company or of a duly authorized transfer agent of the Company), no right to vote or receive dividends or any other rights as a stockholder will exist with respect to the Shares, notwithstanding the exercise of the Option. The Company will issue (or cause to be issued) such Shares promptly after the Option is exercised. No adjustment will be made for a dividend or other right for which the record date is prior to the date the Shares are issued, except as provided in Section 2.6 of the Plan. Exercising an Option in any manner will decrease the number of Shares thereafter available, both for purposes of the Plan and for sale under the Option, by the number of Shares as to which the Option is exercised.

(f) Termination of Service. If the Participant's Service terminates for any reason except for Cause or the Participant's death or Disability, then the Participant may exercise such Participant's Options only to the extent that such Options would have been exercisable by the Participant on the date Participant's Service terminates no later than three (3) months after the date Participant's Service terminates (or such shorter time period not less than thirty (30) days or longer time period as may be determined by the Committee, with any exercise beyond three (3) months after the date Participant's Service terminates deemed to be the exercise of an NSO), but in any event no later than the expiration date of the Options.

(i) Death. If the Participant's Service terminates because of the Participant's death (or the Participant dies within three (3) months after Participant's Service terminates other than for Cause or because of the Participant's Disability), then the Participant's Options may be exercised only to the extent that such Options would have been exercisable by the Participant on the date Participant's Service terminates and must be exercised by the Participant's legal representative, or authorized assignee, no later than twelve (12) months after the date Participant's Service terminates (or such shorter time period not less than six (6) months or longer time period as may be determined by the Committee), but in any event no later than the expiration date of the Options.

(ii) Disability. If the Participant's Service terminates because of the Participant's Disability, then the Participant's Options may be exercised only to the extent that such Options would have been exercisable by the Participant on the date Participant's Service terminates and must be exercised by the Participant (or the Participant's legal representative or authorized assignee) no later than twelve (12) months after the date Participant's Service terminates (or such shorter time period not less than six (6) months or longer time period as may be determined by the Committee, with any exercise beyond (a) three (3) months after the date Participant's Service terminates when the termination of Service is for a Disability that is not a "permanent and total disability" as defined in Section 22(e)(3) of the Code or (b) twelve (12) months after the date Participant's Service terminates when the termination of Service is for a Disability that is a "permanent and total disability" as defined in Section 22(e)(3) of the Code, deemed to be exercise of an NSO), but in any event no later than the expiration date of the Options.

(iii) Cause. Unless determined otherwise by the Committee, if the Participant's Service terminates for Cause, then Participant's Options (whether or not vested) will expire on the date of termination of Participant's Service if the Committee has reasonably determined in good faith that such cessation of Services has resulted in connection with an act or failure to act constituting Cause (or such Participant's Services could have been terminated for Cause (without regard to the lapsing of any required notice or cure periods in connection therewith) at the time such Participant terminated Services), or at such later time and on such conditions as are determined by the Committee, but in any event no later than the expiration date of the Options. Unless otherwise provided in an employment agreement, Award Agreement, or other applicable agreement, Cause will have the meaning set forth in the Plan.

(g) Limitations on Exercise. The Committee may specify a minimum number of Shares that may be purchased on any exercise of an Option, provided that such minimum number will not prevent any Participant from exercising the Option for the full number of Shares for which it is then exercisable.

(h) Limitations on ISOs. With respect to Awards granted as ISOs, to the extent that the aggregate Fair Market Value of the Shares with respect to which such ISOs are exercisable for the first time by the Participant during any calendar year (under all plans of the Company and any Parent or Subsidiary) exceeds one hundred thousand dollars ($100,000), such Options will be treated as NSOs. For purposes of this Section 5.8, ISOs will be taken into account in the order in which they were granted. The Fair Market Value of the Shares will be determined as of the time the Option with respect to such Shares is granted. In the event that the Code or the regulations promulgated thereunder are amended after the Effective Date to provide for a different limit on the Fair Market Value of Shares permitted to be subject to ISOs, such different limit will be automatically incorporated herein and will apply to any Options granted after the effective date of such amendment.

(i) Modification, Extension or Renewal. The Committee may modify, extend, or renew outstanding Options and authorize the grant of new Options in substitution therefor, provided that any such action may not, without the written consent of a Participant, impair any of such Participant's rights under any Option previously granted. Any outstanding ISO that is modified, extended, renewed, or otherwise altered will be treated in accordance with Section 424(h) of the Code. Subject to Section 19 of this Plan, by written notice to affected Participants, the Committee may reduce the Exercise Price of outstanding Options without the consent of such Participants, provided, however, that the Exercise Price may not be reduced below the Fair Market Value on the date the action is taken to reduce the Exercise Price.

(j) No Disqualification. Notwithstanding any other provision in this Plan, no term of this Plan relating to ISOs will be interpreted, amended, or altered, nor will any discretion or authority granted under this Plan be exercised, so as to disqualify this Plan under Section 422 of the Code or, without the consent of the Participant affected, to disqualify any ISO under Section 422 of the Code.

6. RESTRICTED STOCK AWARDS. A Restricted Stock Award is an offer by the Company to sell to an eligible Employee, Consultant, or Director Shares that are subject to restrictions ("***Restricted Stock***"). The Committee will determine to whom an offer will be made, the number of Shares the Participant may purchase, the Purchase Price, the restrictions under which the Shares will be subject, and all other terms and conditions of the Restricted Stock Award, subject to the Plan.

(a) Restricted Stock Purchase Agreement. All purchases under a Restricted Stock Award will be evidenced by an Award Agreement. Except as may otherwise be provided in an Award Agreement, a Participant accepts a Restricted Stock Award by signing and delivering to the Company an Award Agreement with full payment of the Purchase Price, within thirty (30) days from the date the Award Agreement was delivered to the Participant. If the Participant does not accept such Award within thirty (30) days, then the offer of such Restricted Stock Award will terminate, unless the Committee determines otherwise.

(b) Purchase Price. The Purchase Price for a Restricted Stock Award will be determined by the Committee and may be less than Fair Market Value on the date the Restricted Stock Award is granted. Payment of the Purchase Price must be made in accordance with Section 12 of the Plan, and the Award Agreement and in accordance with any procedures established by the Company.

(c) Terms of Restricted Stock Awards. Restricted Stock Awards will be subject to such restrictions as the Committee may impose or are required by law. These restrictions may be based on completion of a specified period of Service with the Company or upon completion of Performance Factors, if any, during any Performance Period as set out in advance in the Participant's Award Agreement. Prior to the grant of a Restricted Stock Award, the Committee will: (a) determine the nature, length, and starting date of any Performance Period for the Restricted Stock Award; (b) select from among the Performance Factors to be used to measure performance goals, if any; and (c) determine the number of Shares that may be awarded to the Participant. Performance Periods may overlap and a Participant may participate simultaneously with respect to Restricted Stock Awards that are subject to different Performance Periods and having different performance goals and other criteria.

(d) Termination of Service. Except as may be set forth in the Participant's Award Agreement, vesting ceases on such date Participant's Service terminates (unless determined otherwise by the Committee).

7. STOCK BONUS AWARDS. A Stock Bonus Award is an award to an eligible Employee, Consultant, or Director of Shares for Services to be rendered or for past Services already rendered to the Company or any Parent, Subsidiary, or Affiliate. All Stock Bonus Awards will be made pursuant to an Award Agreement. No payment from the Participant will be required for Shares awarded pursuant to a Stock Bonus Award.

(a) Terms of Stock Bonus Awards. The Committee will determine the number of Shares to be awarded to the Participant under a Stock Bonus Award and any restrictions thereon. These restrictions may be based upon completion of a specified period of Service with the Company or upon satisfaction of performance goals based on Performance Factors during any Performance Period as set out in advance in the Participant's Stock Bonus Agreement. Prior to the grant of any Stock Bonus Award the Committee will: (a) determine the nature, length, and starting date of any Performance Period for the Stock Bonus Award; (b) select from among the Performance Factors to be used to measure performance goals; and (c) determine the number of Shares that may be awarded to the Participant. Performance Periods may overlap and a Participant may participate simultaneously with respect to Stock Bonus Awards that are subject to different Performance Periods and different performance goals and other criteria.

(b) Form of Payment to Participant. Payment may be made in the form of cash, whole Shares, or a combination thereof, based on the Fair Market Value of the Shares earned under a Stock Bonus Award on the date of payment, as determined in the sole discretion of the Committee.

(c) Termination of Service. Except as may be set forth in the Participant's Award Agreement, vesting ceases on such date Participant's Service terminates (unless determined otherwise by the Committee).

8. STOCK APPRECIATION RIGHTS. A Stock Appreciation Right ("***SAR***") is an award to an eligible Employee, Consultant, or Director that may be settled in cash or Shares (which may consist of Restricted Stock) having a value equal to (a) the difference between the Fair Market Value on the date of exercise over the Exercise Price multiplied by (b) the number of Shares with respect to which the SAR is being settled (subject to any maximum number of Shares that may be issuable as specified in an Award Agreement). All SARs will be made pursuant to an Award Agreement.

(a) Terms of SARs. The Committee will determine the terms of each SAR including, without limitation: (a) the number of Shares subject to the SAR, (b) the Exercise Price and the time or times during which the SAR may be settled, (c) the consideration to be distributed on settlement of the SAR, and (d) the effect of the Participant's termination of Service on each SAR. The Exercise Price of the SAR will be determined by the Committee when the SAR is granted and may not be less than Fair Market Value of the Shares on the date of grant. A SAR may be awarded upon satisfaction of Performance Factors, if any, during any Performance Period as are set out in advance in the Participant's individual Award Agreement. If the SAR is being earned upon the satisfaction of Performance Factors, then the Committee will: (i) determine the nature, length, and starting date of any Performance Period for each SAR; and (ii) select from among the Performance Factors to be used to measure the performance, if any. Performance Periods may overlap and Participants may participate simultaneously with respect to SARs that are subject to different Performance Factors and other criteria.

(b) Exercise Period and Expiration Date. A SAR will be exercisable within the times or upon the occurrence of events determined by the Committee and set forth in the Award Agreement governing such SAR. The SAR Agreement will set forth the expiration date, provided that no SAR will be exercisable after the expiration of ten (10) years from the date the SAR is granted. The Committee may also provide for SARs to become exercisable at one time or from time to time, periodically or otherwise (including, without limitation, upon the attainment during a Performance Period of performance goals based on Performance Factors), in such number of Shares or percentage of the Shares subject to the SAR as the Committee determines. Except as may be set forth in the Participant's Award Agreement, vesting ceases on the date Participant's Service terminates (unless determined otherwise by the Committee). Notwithstanding the foregoing, the rules of Section 5.6 also will apply to SARs.

(c) Form of Settlement. Upon exercise of a SAR, a Participant will be entitled to receive payment from the Company in an amount determined by multiplying (a) the difference between the Fair Market Value of a Share on the date of exercise over the Exercise Price, by (b) the number of Shares with respect to which the SAR is exercised. At the discretion of the Committee, the payment from the Company for the SAR exercise may be in cash, in Shares of equivalent value, or in some combination thereof. The portion of a SAR being settled may be paid currently or on a deferred basis with such interest, if any, as the Committee determines, provided that the terms of the SAR and any deferral satisfy the requirements of Section 409A of the Code to the extent applicable.

(d) Termination of Service. Except as may be set forth in the Participant's Award Agreement, vesting ceases on such date Participant's Service terminates (unless determined otherwise by the Committee).

9. RESTRICTED STOCK UNITS. A Restricted Stock Unit ("***RSU***") is an award to an eligible Employee, Consultant, or Director covering a number of Shares that may be settled in cash, or by issuance of those Shares (which may consist of Restricted Stock). All RSUs will be made pursuant to an Award Agreement.

(a) Terms of RSUs. The Committee will determine the terms of an RSU including, without limitation: (a) the number of Shares subject to the RSU, (b) the time or times during which the RSU may be settled, (c) the consideration to be distributed on settlement, and (d) the effect of the Participant's termination of Service on each RSU, provided that no RSU will have a term longer than ten (10) years. An RSU may be awarded upon satisfaction of such performance goals based on Performance Factors during any Performance Period as are set out in advance in the Participant's Award Agreement. If the RSU is being earned upon satisfaction of Performance Factors, then the Committee will: (i) determine the nature, length, and starting date of any Performance Period for the RSU; (ii) select from among the Performance Factors to be used to measure the performance, if any; and (iii) determine the number of Shares deemed subject to the RSU. Performance Periods may overlap and Participants may participate simultaneously with respect to RSUs that are subject to different Performance Periods and different performance goals and other criteria.

(b) Form and Timing of Settlement. Payment of earned RSUs will be made as soon as practicable after the date(s) determined by the Committee and set forth in the Award Agreement. The Committee, in its sole discretion, may settle earned RSUs in cash, Shares, or a combination of both. The Committee may also permit a Participant to defer payment under a RSU to a date or dates after the RSU is earned, provided that the terms of the RSU and any deferral satisfy the requirements of Section 409A of the Code to the extent applicable.

(c) Termination of Service. Except as may be set forth in the Participant's Award Agreement, vesting ceases on such date Participant's Service terminates (unless determined otherwise by the Committee).

10. PERFORMANCE AWARDS.

(a) Types of Performance Awards. A Performance Award is an award to an eligible Employee, Consultant, or Director of the Company or any Parent, Subsidiary, or Affiliate that is based upon the attainment of performance goals, as established by the Committee, and other terms and conditions specified by the Committee, and may be settled in cash, Shares (which may consist of, without limitation, Restricted Stock), other property, or any combination thereof. Grants of Performance Awards will be made pursuant to an Award Agreement.

(i) Performance Shares. The Committee may grant Awards of Performance Shares, designate the Participants to whom Performance Shares are to be awarded, and determine the number of Performance Shares and the terms and conditions of each such Award. Performance Shares will consist of a unit valued by reference to a designated number of Shares, the value of which may be paid to the Participant by delivery of Shares or, if set forth in the instrument evidencing the Award, of such property as the Committee will determine, including, without limitation, cash, Shares, other property, or any combination thereof, upon the attainment of performance goals, as established by the Committee, and other terms and conditions specified by the Committee. The amount to be paid under an Award of Performance Shares may be adjusted on the basis of such further consideration as the Committee will determine in its sole discretion.

(ii) Performance Units. The Committee may grant Awards of Performance Units, designate the Participants to whom Performance Units are to be awarded, and determine the number of Performance Units and the terms and conditions of each such Award. Performance Units will consist of

a unit valued by reference to a designated amount of property other than Shares, which value may be paid to the Participant by delivery of such property as the Committee will determine, including, without limitation, cash, Shares, other property, or any combination thereof, upon the attainment of performance goals, as established by the Committee, and other terms and conditions specified by the Committee.

(iii) Cash-Settled Performance Awards. The Committee may also grant cash-based Performance Awards to Participants under the terms of this Plan. Such awards will be based on the attainment of performance goals using the Performance Factors within this Plan that are established by the Committee for the relevant performance period.

(b) Terms of Performance Awards. The Committee will determine, and each Award Agreement will set forth, the terms of each Performance Award including, without limitation: (a) the amount of any cash bonus, (b) the number of Shares deemed subject to an award of Performance Shares, (c) the Performance Factors and Performance Period that will determine the time and extent to which each award of Performance Shares will be settled, (d) the consideration to be distributed on settlement, and (e) the effect of the Participant's termination of Service on each Performance Award. In establishing Performance Factors and the Performance Period the Committee will: (i) determine the nature, length, and starting date of any Performance Period; (ii) select from among the Performance Factors to be used; and (iii) determine the number of Shares deemed subject to the award of Performance Shares. Each Performance Share will have an initial value equal to the Fair Market Value of a Share on the date of grant. Prior to settlement the Committee will determine the extent to which Performance Awards have been earned. Performance Periods may overlap and Participants may participate simultaneously with respect to Performance Awards that are subject to different Performance Periods and different performance goals and other criteria.

(c) Termination of Service. Except as may be set forth in the Participant's Award Agreement, vesting ceases on the date Participant's Service terminates (unless determined otherwise by the Committee).

11. CASH AWARDS. A Cash Award ("***Cash Award***") is an award that is denominated in, or payable to an eligible Participant solely in, cash, as deemed by the Committee to be consistent with the purposes of the Plan. Cash Awards shall be subject to the terms, conditions, restrictions and limitations determined by the Committee, in its sole discretion, from time to time. Awards granted pursuant to this Section 11 may be granted with value and payment contingent upon the achievement of Performance Factors.

12. PAYMENT FOR SHARE PURCHASES. Payment from a Participant for Shares purchased pursuant to this Plan may be made in cash or by check or, where expressly approved for the Participant by the Committee and where permitted by law (and to the extent not otherwise set forth in the applicable Award Agreement):

(i) by cancellation of indebtedness of the Company to the Participant;

(ii) by surrender of shares of the Company held by the Participant that have a Fair Market Value on the date of surrender equal to the aggregate exercise price of the Shares as to which said Award will be exercised or settled;

(iii) by waiver of compensation due or accrued to the Participant for services rendered or to be rendered to the Company or a Parent or Subsidiary;

(iv) by consideration received by the Company pursuant to a broker-assisted or other form of cashless exercise program implemented by the Company in connection with the Plan;

(v) by any combination of the foregoing; or

(vi) by any other method of payment as is permitted by applicable law.

The Committee may limit the availability of any method of payment, to the extent the Committee determines, in its discretion, such limitation is necessary or advisable to comply with applicable law or facilitate the administration of the Plan.

13. GRANTS TO NON-EMPLOYEE DIRECTORS.

(a) General. Non-Employee Directors are eligible to receive any type of Award offered under this Plan except ISOs. Awards pursuant to this Section 13 may be automatically made pursuant to policy adopted by the Board, or made from time to time as determined in the discretion of the Board. No Non-Employee Director may receive Awards under the Plan that, when combined with cash compensation received for service as a Non-Employee Director, exceed five hundred thousand ($500,000) in value (as described below) in any calendar year, increased to one million ($1,000,000) in value (as described below) in the calendar year of his or her initial services as a Non-Employee Director. The value of Awards for purposes of complying with this maximum will be determined as follows: (a) for Options and SARs, grant date fair value will be calculated using the Black-Scholes valuation methodology on the date of grant of such Option or SAR, and (b) for all other Awards other than Options and SARs, grant date fair value will be determined by either (i) calculating the product of the Fair Market Value per Share on the date of grant and the aggregate number of Shares subject to the Award, or (ii) calculating the product using an average of the Fair Market Value over a number of trading days and the aggregate number of Shares subject to the Award as determined by the Committee. Awards granted to an individual while he or she was serving in the capacity as an Employee or while he or she was a Consultant but not a Non-Employee Director will not count for purposes of the limitations set forth in this Section 13.1.

(b) Eligibility. Awards pursuant to this Section 13 will be granted only to Non-Employee Directors. A Non-Employee Director who is elected or re-elected as a member of the Board will be eligible to receive an Award under this Section 13.

(c) Vesting, Exercisability and Settlement. Except as set forth in Section 22, Awards will vest, become exercisable, and be settled as determined by the Board. With respect to Options and SARs, the exercise price granted to Non-Employee Directors will not be less than the Fair Market Value of the Shares at the time that such Option or SAR is granted.

(d) Election to Receive Awards in Lieu of Cash. A Non-Employee Director may elect to receive his or her annual retainer payments and/or meeting fees from the Company in the form of cash or Awards or a combination thereof, if permitted, and as determined, by the Committee. Such Awards will be issued under the Plan. An election under this Section 13.4 will be filed with the Company on the form prescribed by the Company.

14. WITHHOLDING TAXES.

(a) Withholding Generally. Whenever Shares are to be issued in satisfaction of Awards granted under this Plan or a tax event occurs, the Company may require the Participant to remit to the Company, or to the Parent, Subsidiary, or Affiliate, as applicable, employing the Participant an amount sufficient to satisfy applicable U.S. federal, state, local, and international tax or any other tax or social insurance liability (the "***Tax-Related Items***") legally due from the Participant prior to the delivery of Shares pursuant to exercise or settlement of any Award. Whenever payments in satisfaction of Awards granted under this Plan are to be made in cash, such payment will be net of an amount sufficient to satisfy applicable withholding obligations for Tax-Related Items. Unless otherwise determined by the Committee, the Fair

Market Value of the Shares will be determined as of the date that the taxes are required to be withheld and such Shares will be valued based on the value of the actual trade or, if there is none, the Fair Market Value of the Shares as of the previous trading day.

(b) Stock Withholding. The Committee, or its delegate(s), as permitted by applicable law, in its sole discretion and pursuant to such procedures as it may specify from time to time and to limitations of local law, may require or permit a Participant to satisfy such Tax Related Items legally due from the Participant, in whole or in part by (without limitation) (a) paying cash, (b) having the Company withhold otherwise deliverable cash or Shares having a Fair Market Value equal to the Tax-Related Items to be withheld, (c) delivering to the Company already-owned shares having a Fair Market Value equal to the Tax-Related Items to be withheld, or (d) withholding from the proceeds of the sale of otherwise deliverable Shares acquired pursuant to an Award either through a voluntary sale or through a mandatory sale arranged by the Company. The Company may withhold or account for these Tax-Related Items by considering applicable statutory withholding rates or other applicable withholding rates, including up to the maximum permissible statutory tax rate for the applicable tax jurisdiction, to the extent consistent with applicable laws.

15. TRANSFERABILITY. Unless determined otherwise by the Committee, an Award may not be sold, pledged, assigned, hypothecated, transferred, or disposed of in any manner other than by will or by the laws of descent or distribution. If the Committee makes an Award transferable, including, without limitation, by instrument to an inter vivos or testamentary trust in which the Awards are to be passed to beneficiaries upon the death of the trustor (settlor) or by gift or by domestic relations order to a Permitted Transferee, such Award will contain such additional terms and conditions as the Committee deems appropriate. All Awards will be exercisable: (a) during the Participant's lifetime only by the Participant or the Participant's guardian or legal representative; (b) after the Participant's death, by the legal representative of the Participant's heirs or legatees; and (c) in the case of all awards except ISOs, by a Permitted Transferee.

16. PRIVILEGES OF STOCK OWNERSHIP; RESTRICTIONS ON SHARES.

(a) Voting and Dividends. No Participant will have any of the rights of a stockholder with respect to any Shares until the Shares are issued to the Participant, except for any Dividend Equivalent Rights permitted by an applicable Award Agreement. Any Dividend Equivalent Rights will be subject to the same vesting or performance conditions as the underlying Award. In addition, the Committee may provide that any Dividend Equivalent Rights permitted by an applicable Award Agreement will be deemed to have been reinvested in additional Shares or otherwise reinvested. After Shares are issued to the Participant, the Participant will be a stockholder and have all the rights of a stockholder with respect to such Shares, including the right to vote and receive all dividends or other distributions made or paid with respect to such Shares; provided, that if such Shares are Restricted Stock, then any new, additional or different securities the Participant may become entitled to receive with respect to such Shares by virtue of a stock dividend, stock split or any other change in the corporate or capital structure of the Company will be subject to the same restrictions as the Restricted Stock; provided, further, that the Participant will have no right to such stock dividends or stock distributions with respect to Unvested Shares, and any such dividends or stock distributions will be accrued and paid only at such time, if any, as such Unvested Shares become vested Shares. The Committee, in its discretion, may provide in the Award Agreement evidencing any Award that the Participant will be entitled to Dividend Equivalent Rights with respect to the payment of cash dividends on Shares underlying an Award during the period beginning on the date the Award is granted and ending, with respect to each Share subject to the Award, on the earlier of the date on which the Award is exercised or settled or the date on which it is forfeited; provided, that no Dividend Equivalent Right will be paid with respect to the Unvested Shares, and such dividends or stock distributions will be accrued and paid only at such time, if any, as such Unvested Shares become vested Shares. Such Dividend Equivalent Rights, if any, will be credited to the Participant in the form of additional whole Shares as of the date of payment of such cash dividends on Shares.

(b) Restrictions on Shares. At the discretion of the Committee, the Company may reserve to itself and/or its assignee(s) a right to repurchase (a "***Right of Repurchase***") a portion of any or all Unvested Shares held by a Participant following such Participant's termination of Service at any time within ninety (90) days (or such longer or shorter time determined by the Committee) after the later of the date Participant's Service terminates and the date the Participant purchases Shares under this Plan, for cash and/or cancellation of purchase money indebtedness, at the Participant's Purchase Price or Exercise Price, as the case may be.

17. CERTIFICATES. All Shares or other securities whether or not certificated, delivered under this Plan will be subject to such stock transfer orders, legends, and other restrictions as the Committee may deem necessary or advisable, including restrictions under any applicable U.S. federal, state, or foreign securities law, or any rules, regulations, and other requirements of the SEC or any stock exchange or automated quotation system upon which the Shares may be listed or quoted, and any non-U.S. exchange controls or securities law restrictions to which the Shares are subject.

18. ESCROW; PLEDGE OF SHARES. To enforce any restrictions on a Participant's Shares, the Committee may require the Participant to deposit all certificates representing Shares, together with stock powers or other instruments of transfer approved by the Committee, appropriately endorsed in blank, with the Company or an agent designated by the Company to hold in escrow until such restrictions have lapsed or terminated, and the Committee may cause a legend or legends referencing such restrictions to be placed on the certificates. Any Participant who is permitted to execute a promissory note as partial or full consideration for the purchase of Shares under this Plan will be required to pledge and deposit with the Company all or part of the Shares so purchased as collateral to secure the payment of the Participant's obligation to the Company under the promissory note, provided, however, that the Committee may require or accept other or additional forms of collateral to secure the payment of such obligation and, in any event, the Company will have full recourse against the Participant under the promissory note notwithstanding any pledge of the Participant's Shares or other collateral. In connection with any pledge of the Shares, the Participant will be required to execute and deliver a written pledge agreement in such form as the Committee will from time to time approve. The Shares purchased with the promissory note may be released from the pledge on a pro rata basis as the promissory note is paid.

19. REPRICING; EXCHANGE AND BUYOUT OF AWARDS. Without prior stockholder approval the Committee may (a) reprice Options or SARs (and where such repricing is a reduction in the Exercise Price of outstanding Options or SARs, the consent of the affected Participants is not required provided written notice is provided to them, notwithstanding any adverse tax consequences to them arising from the repricing), and (b) with the consent of the respective Participants (unless not required pursuant to Section 5.9 of the Plan), pay cash or issue new Awards in exchange for the surrender and cancellation of any, or all, outstanding Awards.

20. SECURITIES LAW AND OTHER REGULATORY COMPLIANCE. An Award will not be effective unless such Award is in compliance with all applicable U.S. and foreign federal and state securities and exchange control and other laws, rules, and regulations of any governmental body, and the requirements of any stock exchange or automated quotation system upon which the Shares may then be listed or quoted, as they are in effect on the date of grant of the Award and also on the date of exercise or other issuance. Notwithstanding any other provision in this Plan, the Company will have no obligation to issue or deliver certificates for Shares under this Plan prior to: (a) obtaining any approvals from governmental agencies that the Company determines are necessary or advisable and/or (b) completion of any registration or other qualification of such Shares under any state, federal, or foreign law or ruling of

any governmental body that the Company determines to be necessary or advisable. The Company will be under no obligation to register the Shares with the SEC or to effect compliance with the registration, qualification, or listing requirements of any foreign or state securities laws, exchange control laws, stock exchange, or automated quotation system, and the Company will have no liability for any inability or failure to do so.

21. NO OBLIGATION TO EMPLOY. Nothing in this Plan or any Award granted under this Plan will confer or be deemed to confer on any Participant any right to continue in the employ of, or to continue any other relationship with, the Company or any Parent, Subsidiary, or Affiliate or limit in any way the right of the Company or any Parent, Subsidiary, or Affiliate to terminate Participant's employment or other relationship at any time.

22. CORPORATE TRANSACTIONS.

(a) Assumption or Replacement of Awards by Successor. In the event of a Corporate Transaction any or all outstanding Awards may be assumed, converted, replaced, or substituted by the successor corporation, which assumption, conversion, replacement or substitution will be binding on all Participants. In the event of a substitution, the successor corporation may substitute equivalent Awards or provide substantially similar consideration to Participants as was provided to stockholders (after taking into account the existing provisions of the Awards). The successor corporation may also issue, as replacement of outstanding Shares of the Company held by the Participant, substantially similar shares or other property subject to repurchase restrictions no less favorable to the Participant. In the event such successor or acquiring corporation (if any) refuses to assume, convert, replace or substitute Awards, as provided above, pursuant to a Corporate Transaction, then notwithstanding any other provision in this Plan to the contrary, such Awards shall have their vesting accelerate as to all Shares or cash subject to such Awards (and any applicable right of repurchase shall fully lapse) immediately prior to the Corporate Transaction and all such Awards shall expire on such Corporate Transaction at such time and on such conditions as the Board will determine. In addition, in the event such successor or acquiring corporation (if any) refuses to assume, convert, replace, or substitute Awards, as provided above, pursuant to a Corporate Transaction, the Committee will notify the Participant in writing or electronically that such Participant's Award will, if exercisable, be exercisable for a period of time determined by the Committee in its sole discretion, and such Award will terminate upon the expiration of such period. Awards need not all be treated in the same manner in a Corporate Transaction, and treatment may vary from Award to Award and/or from Participant to Participant.

(b) Assumption of Awards by the Company. The Company, from time to time, also may substitute or assume outstanding awards granted by another company, whether in connection with an acquisition of such other company or otherwise, by either: (a) granting an Award under this Plan in substitution of such other company's award, or (b) assuming such award as if it had been granted under this Plan if the terms of such assumed award could be applied to an Award granted under this Plan. Such substitution or assumption will be permissible if the holder of the substituted or assumed award would have been eligible to be granted an Award under this Plan if the other company had applied the rules of this Plan to such grant. In the event the Company assumes an award granted by another company, the terms and conditions of such award will remain unchanged (except that the Purchase Price or the Exercise Price, as the case may be, and the number and nature of Shares issuable upon exercise or settlement of any such Award will be adjusted appropriately pursuant to Section 424(a) of the Code). In the event the Company elects to grant a new Option in substitution rather than assuming an existing option, such new Option may be granted with a similarly adjusted Exercise Price. Substitute Awards will not reduce the number of Shares authorized for grant under the Plan or authorized for grant to a Participant in a calendar year.

(c) Non-Employee Directors' Awards. Notwithstanding any provision to the contrary herein, in the event of a Corporate Transaction, the vesting of all Awards granted to Non-Employee Directors will accelerate and such Awards will become exercisable (as applicable) in full prior to the consummation of such event at such times and on such conditions as the Committee determines.

23. **ADOPTION AND STOCKHOLDER APPROVAL**. This Plan will be submitted for the approval of the Company's stockholders, consistent with applicable laws, within twelve (12) months before or after the date this Plan is adopted by the Board.

24. **TERM OF PLAN/GOVERNING LAW**. Unless earlier terminated as provided herein, this Plan will become effective on the Effective Date and will terminate ten (10) years from the date this Plan is adopted by the Board. This Plan and all Awards granted hereunder will be governed by and construed in accordance with the laws of the State of Delaware (excluding its conflict of laws rules).

25. **AMENDMENT OR TERMINATION OF PLAN**. The Board may at any time terminate or amend this Plan in any respect, including, without limitation, amendment of any form of Award Agreement or instrument to be executed pursuant to this Plan, provided, however, that the Board will not, without the approval of the stockholders of the Company, amend this Plan in any manner that requires such stockholder approval, provided further that a Participant's Award will be governed by the version of this Plan then in effect at the time such Award was granted. No termination or amendment of the Plan will affect any then-outstanding Award unless expressly provided by the Committee. In any event, no termination or amendment of the Plan or any outstanding Award may adversely affect any then outstanding Award without the consent of the Participant, unless such termination or amendment is necessary to comply with applicable law, regulation, or rule.

26. **NONEXCLUSIVITY OF THE PLAN**. Neither the adoption of this Plan by the Board, the submission of this Plan to the stockholders of the Company for approval, nor any provision of this Plan will be construed as creating any limitations on the power of the Board to adopt such additional compensation arrangements as it may deem desirable, including, without limitation, the granting of stock awards and bonuses otherwise than under this Plan, and such arrangements may be either generally applicable or applicable only in specific cases.

27. **INSIDER TRADING POLICY**. Each Participant who receives an Award will comply with any policy adopted by the Company from time to time covering transactions in the Company's securities by Employees, officers, and/or Directors of the Company, as well as with any applicable insider trading or market abuse laws to which the Participant may be subject.

28. **ALL AWARDS SUBJECT TO COMPANY CLAWBACK OR RECOUPMENT POLICY**. All Awards, subject to applicable law, will be subject to clawback or recoupment pursuant to any compensation clawback or recoupment policy adopted by the Board or required by law during the term of Participant's employment or other service with the Company that is applicable to officers, Employees, Directors or other service providers of the Company, and in addition to any other remedies available under such policy and applicable law, may require the cancellation of outstanding Awards and the recoupment of any gains realized with respect to Awards.

29. **DEFINITIONS**. As used in this Plan, and except as elsewhere defined herein, the following terms will have the following meanings:

(a) "***Affiliate***" means (a) any entity that, directly or indirectly, is controlled by, controls, or is under common control with, the Company, and (b) any entity in which the Company has a significant equity interest, in either case as determined by the Committee, whether now or hereafter existing.

(b) "***Award***" means any award under the Plan, including any Option, Performance Award, Cash Award, Restricted Stock, Stock Bonus, Stock Appreciation Right, or Restricted Stock Unit.

(c) "***Award Agreement***" means, with respect to each Award, the written or electronic agreement between the Company and the Participant setting forth the terms and conditions of the Award, and country-specific appendix thereto for grants to non-U.S. Participants, which will be in substantially a form (which need not be the same for each Participant) that the Committee (or in the case of Award agreements that are not used for Insiders, the Committee's delegate(s)) has from time to time approved, and will comply with and be subject to the terms and conditions of this Plan.

(d) "***Board***" means the Board of Directors of the Company.

(e) "***Cash Award***" means an award as defined in Section 11 and granted under the Plan.

(f) "***Cause***" means a determination by the Company that the Participant has committed an act or acts constituting any of the following: (i) dishonesty, fraud, misconduct or negligence in connection with Participant's duties to the Company, (ii) unauthorized disclosure or use of the Company's confidential or proprietary information, (iii) misappropriation of a business opportunity of the Company, (iv) materially aiding Company competitor, (v) a felony conviction, (vi) failure or refusal to attend to the duties or obligations of the Participant's position (vii) violation or breach of, or failure to comply with, the Company's code of ethics or conduct, any of the Company's rules, policies or procedures applicable to the Participant or any agreement in effect between the Company and the Participant or (viii) other conduct by such Participant that could be expected to be harmful to the business, interests or reputation of the Company. The determination as to whether Cause for a Participant's termination exists will be made in good faith by the Company and will be final and binding on the Participant. This definition does not in any way limit the Company's or any Parent's or Subsidiary's ability to terminate a Participant's employment or services at any time as provided in Section 21 above. Notwithstanding the foregoing, the foregoing definition of "Cause" may, in part or in whole, be modified or replaced in each individual employment agreement, Award Agreement, or other applicable agreement with any Participant, provided that such document supersedes the definition provided in this Section 29.6.

(g) "***Code***" means the United States Internal Revenue Code of 1986, as amended, and the regulations promulgated thereunder.

(h) "***Committee***" means the Compensation Committee of the Board or those persons to whom administration of the Plan, or part of the Plan, has been delegated as permitted by law.

(i) "***Common Stock***" means the common stock of the Company.

(j) "***Company***" means Stoke Therapeutics, Inc., a Delaware corporation, or any successor corporation.

(k) "***Consultant***" means any natural person, including an advisor or independent contractor, engaged by the Company or a Parent, Subsidiary, or Affiliate to render services to such entity.

(l) "***Corporate Transaction***" means the occurrence of any of the following events: (a) any "Person" (as such term is used in Sections 13(d) and 14(d) of the Exchange Act) becomes the "beneficial owner" (as defined in Rule 13d-3 of the Exchange Act), directly or indirectly, of securities of the Company representing more than fifty percent (50%) of the total voting power represented by the Company's then-outstanding voting securities, provided, however, that for purposes of this subclause (a) the acquisition of additional securities by any one Person who is considered to own more than fifty percent (50%) of the total

voting power of the securities of the Company will not be considered a Corporate Transaction; (b) the consummation of the sale or disposition by the Company of all or substantially all of the Company's assets; (c) the consummation of a merger or consolidation of the Company with any other corporation, other than a merger or consolidation which would result in the voting securities of the Company outstanding immediately prior thereto continuing to represent (either by remaining outstanding or by being converted into voting securities of the surviving entity or its parent) at least fifty percent (50%) of the total voting power represented by the voting securities of the Company or such surviving entity or its parent outstanding immediately after such merger or consolidation; (d) any other transaction which qualifies as a "corporate transaction" under Section 424(a) of the Code wherein the stockholders of the Company give up all of their equity interest in the Company (except for the acquisition, sale or transfer of all or substantially all of the outstanding shares of capital stock of the Company), or (e) a change in the effective control of the Company that occurs on the date that a majority of members of the Board is replaced during any twelve (12) month period by members of the Board whose appointment or election is not endorsed by a majority of the members of the Board prior to the date of the appointment or election. For purpose of this subclause (e), if any Person is considered to be in effective control of the Company, the acquisition of additional control of the Company by the same Person will not be considered a Corporate Transaction. For purposes of this definition, Persons will be considered to be acting as a group if they are owners of a corporation that enters into a merger, consolidation, purchase, or acquisition of stock, or similar business transaction with the Company. Notwithstanding the foregoing, to the extent that any amount constituting deferred compensation (as defined in Section 409A of the Code) would become payable under this Plan by reason of a Corporate Transaction, such amount will become payable only if the event constituting a Corporate Transaction would also qualify as a change in ownership or effective control of the Company or a change in the ownership of a substantial portion of the assets of the Company, each as defined within the meaning of Code Section 409A, as it has been and may be amended from time to time, and any proposed or final Treasury Regulations and IRS guidance that has been promulgated or may be promulgated thereunder from time to time.

(m) "***Director***" means a member of the Board.

(n) "***Disability***" means in the case of incentive stock options, total and permanent disability as defined in Section 22(e)(3) of the Code and in the case of other Awards, that the Participant is unable to engage in any substantial gainful activity by reason of any medically determinable physical or mental impairment that can be expected to result in death or can be expected to last for a continuous period of not less than twelve (12) months.

(o) "***Dividend Equivalent Right***" means the right of a Participant, granted at the discretion of the Committee or as otherwise provided by the Plan, to receive a credit for the account of such Participant in an amount equal to the cash, stock, or other property dividends in amounts equal equivalent to cash, stock, or other property dividends for each Share represented by an Award held by such Participant.

(p) "***Effective Date***" means the day immediately prior to the Company's IPO Registration Date, subject to approval of the Plan by the Company's stockholders.

(q) "***Employee***" means any person, including officers and Directors, providing services as an employee to the Company or any Parent, Subsidiary, or Affiliate. Neither service as a Director nor payment of a director's fee by the Company will be sufficient to constitute "employment" by the Company.

(r) "***Exchange Act***" means the United States Securities Exchange Act of 1934, as amended.

(s) "***Exchange Program***" means a program pursuant to which (a) outstanding Awards are surrendered, cancelled, or exchanged for cash, the same type of Award, or a different Award (or combination thereof); or (b) the exercise price of an outstanding Award is increased or reduced.

(t) "***Exercise Price***" means, with respect to an Option, the price at which a holder may purchase the Shares issuable upon exercise of an Option and with respect to a SAR, the price at which the SAR is granted to the holder thereof.

(u) "***Fair Market Value***" means, as of any date, the value of a Share, determined as follows:

(i) if such Common Stock is publicly traded and is then listed on a national securities exchange, its closing price on the date of determination on the principal national securities exchange on which the Common Stock is listed or admitted to trading as reported in *The Wall Street* Journal or such other source as the Committee deems reliable;

(ii) if such Common Stock is publicly traded but is neither listed nor admitted to trading on a national securities exchange, the average of the closing bid and asked prices on the date of determination as reported in *The Wall Street Journal* or such other source as the Committee deems reliable;

(iii) in the case of an Option or SAR grant made on the IPO Registration Date, the price per share at which Shares are initially offered for sale to the public by the Company's underwriters in the initial public offering of Shares as set forth in the Company's final prospectus included within the registration statement on Form S-1 filed with the SEC under the Securities Act; or

(iv) by the Board or the Committee in good faith.

(v) "***Insider***" means an officer or Director of the Company or any other person whose transactions in the Company's Common Stock are subject to Section 16 of the Exchange Act.

(w) "***IPO Registration Date***" means the date on which the Company's registration statement on Form S-1 in connection with its initial public offering of common stock is declared effective by the SEC under the Securities Act.

(x) "***IRS***" means the United States Internal Revenue Service.

(y) "***Non-Employee Director***" means a Director who is not an Employee of the Company or any Parent, Subsidiary, or Affiliate.

(z) "***Option***" means an award of an option to purchase Shares pursuant to Section 5.

(aa) "***Parent***" means any corporation (other than the Company) in an unbroken chain of corporations ending with the Company if each of such corporations other than the Company owns stock possessing fifty percent (50%) or more of the total combined voting power of all classes of stock in one of the other corporations in such chain.

(bb) "***Participant***" means a person who holds an Award under this Plan.

(cc) "***Performance Award***" means an Award as defined in Section 10 and granted under the Plan, the payment of which is contingent upon achieving certain performance goals established by the Committee.

(dd) "***Performance Factors***" means any of the factors selected by the Committee and specified in an Award Agreement, from among the following measures, either individually, alternatively or in any combination, applied to the Company as a whole or any business unit or Subsidiary, either individually, alternatively, or in any combination, on a GAAP or non-GAAP basis, and measured, to the extent applicable on an absolute basis or relative to a pre-established target, to determine whether the performance goals established by the Committee with respect to applicable Awards have been satisfied:

(i) profit before tax;

(ii) billings;

(iii) revenue;

(iv) net revenue;

(v) earnings (which may include earnings before interest and taxes, earnings before taxes, net earnings, stock-based compensation expenses, depreciation, and amortization);

(vi) operating income;

(vii) operating margin;

(viii) operating profit;

(ix) controllable operating profit or net operating profit;

(x) net profit;

(xi) gross margin;

(xii) operating expenses or operating expenses as a percentage of revenue;

(xiii) net income;

(xiv) earnings per share;

(xv) total stockholder return;

(xvi) market share;

(xvii) return on assets or net assets;

(xviii) the Company's stock price;

(xix) growth in stockholder value relative to a pre-determined index;

(xx) return on equity;

(xxi) return on invested capital;

(xxii) cash flow (including free cash flow or operating cash flows);

(xxiii) cash conversion cycle;

(xxiv) economic value added;

(xxv) individual confidential business objectives;

(xxvi) contract awards or backlog;

(xxvii) overhead or other expense reduction;

(xxviii) credit rating;

(xxix) strategic plan development and implementation;

(xxx) succession plan development and implementation;

(xxxi) improvement in workforce diversity;

(xxxii) customer indicators and/or satisfaction;

(xxxiii) new product invention or innovation;

(xxxiv) attainment of research and development milestones;

(xxxv) improvements in productivity;

(xxxvi) bookings;

(xxxvii) attainment of objective operating goals and employee metrics;

(xxxviii) sales;

(xxxix) expenses;

(xl) balance of cash, cash equivalents, and marketable securities;

(xli) completion of an identified special project;

(xlii) completion of a joint venture or other corporate transaction;

(xliii) employee satisfaction and/or retention;

(xliv) research and development expenses;

(xlv) working capital targets and changes in working capital; and

(xlvi) any other metric that is capable of measurement as determined by the Committee.

The Committee may provide for one or more equitable adjustments to the Performance Factors to preserve the Committee's original intent regarding the Performance Factors at the time of the initial award grant, such as but not limited to, adjustments in recognition of unusual or non-recurring items such as acquisition related activities or changes in applicable accounting rules. It is within the sole discretion of the Committee to make or not make any such equitable adjustments.

(ee) "***Performance Period***" means one or more periods of time, which may be of varying and overlapping durations, as the Committee may select, over which the attainment of one or more Performance Factors will be measured for the purpose of determining a Participant's right to, and the payment of, a Performance Award.

(ff) "***Performance Share***" means an Award as defined in Section 10 and granted under the Plan, the payment of which is contingent upon achieving certain performance goals established by the Committee.

(gg) "***Performance Unit***" means an Award as defined in Section 10 and granted under the Plan, the payment of which is contingent upon achieving certain performance goals established by the Committee.

(hh) "***Permitted Transferee***" means any child, stepchild, grandchild, parent, stepparent, grandparent, spouse, former spouse, sibling, niece, nephew, mother-in-law, father-in-law, son-in-law, daughter-in-law, brother-in-law, or sister-in-law (including adoptive relationships) of the Employee, any person sharing the Employee's household (other than a tenant or employee), a trust in which these persons (or the Employee) have more than 50% of the beneficial interest, a foundation in which these persons (or the Employee) control the management of assets, and any other entity in which these persons (or the Employee) own more than 50% of the voting interests.

(ii) "***Plan***" means this Stoke Therapeutics, Inc., 2019 Equity Incentive Plan.

(jj) "***Purchase Price***" means the price to be paid for Shares acquired under the Plan, other than Shares acquired upon exercise of an Option or SAR.

(kk) "***Restricted Stock Award***" means an Award as defined in Section 6 and granted under the Plan, or issued pursuant to the early exercise of an Option.

(ll) "***Restricted Stock Unit***" means an Award as defined in Section 9 and granted under the Plan.

(mm) "***SEC***" means the United States Securities and Exchange Commission.

(nn) "***Securities Act***" means the United States Securities Act of 1933, as amended.

(oo) "***Service***" will mean service as an Employee, Consultant, Director, or Non-Employee Director, to the Company or a Parent, Subsidiary, or Affiliate, subject to such further limitations as may be set forth in the Plan or the applicable Award Agreement. An Employee will not be deemed to have ceased to provide Service in the case of (a) sick leave, (b) military leave, or (c) any other leave of absence approved by the Company, provided that such leave is for a period of not more than ninety (90) days unless reemployment upon the expiration of such leave is guaranteed by contract or statute. Notwithstanding anything to the contrary, an Employee will not be deemed to have ceased to provide Service if a formal policy adopted from time to time by the Company and issued and promulgated to employees in writing provides otherwise. In the case of any Employee on an approved leave of absence or a reduction in hours worked (for illustrative purposes only, a change in schedule from that of full-time to part-time), the Committee may make such provisions respecting suspension or modification of vesting of the Award while on leave from the employ of the Company or a Parent, Subsidiary, or Affiliate or during such change in

working hours as it may deem appropriate, except that in no event may an Award be exercised after the expiration of the term set forth in the applicable Award Agreement. In the event of military or other protected leave, if required by applicable laws, vesting will continue for the longest period that vesting continues under any other statutory or Company approved leave of absence and, upon a Participant's returning from military leave, he or she will be given vesting credit with respect to Awards to the same extent as would have applied had the Participant continued to provide Service to the Company throughout the leave on the same terms as he or she was providing Service immediately prior to such leave. An employee will have terminated employment as of the date he or she ceases to provide Service (regardless of whether the termination is in breach of local employment laws or is later found to be invalid) and employment will not be extended by any notice period or garden leave mandated by local law, provided, however, that a change in status from an Employee to a Consultant or Non-Employee Director (or vice versa) will not terminate the Participant's Service, unless determined by the Committee, in its discretion. The Committee will have sole discretion to determine whether a Participant has ceased to provide Service and the effective date on which the Participant ceased to provide Service.

(pp) "***Shares***" means shares of the Common Stock and the common stock of any successor entity of the Company.

(qq) "***Stock Appreciation Right***" means an Award defined in Section 8 and granted under the Plan.

(rr) "***Stock Bonus***" means an Award defined in Section 7 and granted under the Plan.

(ss) "***Subsidiary***" means any corporation (other than the Company) in an unbroken chain of corporations beginning with the Company if each of the corporations other than the last corporation in the unbroken chain owns stock possessing fifty percent (50%) or more of the total combined voting power of all classes of stock in one of the other corporations in such chain.

(tt) "***Treasury Regulations***" means regulations promulgated by the United States Treasury Department.

(uu) "***Unvested Shares***" means Shares that have not yet vested or are subject to a right of repurchase in favor of the Company (or any successor thereto).

STOKE THERAPEUTICS, INC.
2019 EQUITY INCENTIVE PLAN
NOTICE OF STOCK OPTION GRANT

Unless otherwise defined herein, the terms defined in the Stoke Therapeutics, Inc. (the "***Company***") 2019 Equity Incentive Plan (the "***Plan***") will have the same meanings in this Notice of Stock Option Grant and the electronic representation of this Notice of Stock Option Grant established and maintained by the Company or a third party designated by the Company (this "***Notice***").

Name:

Address:

You (the "***Participant***") have been granted an option to purchase shares of Common Stock of the Company (the "***Option***") under the Plan subject to the terms and conditions of the Plan, this Notice, and the Stock Option Award Agreement (the "***Option Agreement***"), including any applicable country-specific provisions in any appendix attached hereto (the "***Appendix***"), which constitutes part of the Option Agreement.

Grant Number:

Date of Grant:

Vesting Commencement Date:

Exercise Price per Share:

Total Number of Shares:

Type of Option: Non-Qualified Stock Option

Incentive Stock Option

Expiration Date: , 20 ; the Option expires earlier if Participant's Service terminates earlier, as described in the Option Agreement.

Vesting Schedule: Subject to the limitations set forth in this Notice, the Plan, and the Agreement, the Option will vest in accordance with the following schedule: [*insert applicable vesting schedule*]

By accepting (whether in writing, electronically, or otherwise) the Option, Participant acknowledges and agrees to the following:

1) Participant understands that Participant's Service with the Company or a Parent, Subsidiary, or Affiliate is for an unspecified duration, can be terminated at any time (*i.e.*, is "at-will") except where otherwise prohibited by applicable law, and that nothing in this Notice, the Option Agreement, or the Plan changes the nature of that relationship. Participant acknowledges that the vesting of the Option pursuant to this Notice is subject to Participant's continuing Service as an Employee, Director, or Consultant. Participant agrees and acknowledges that the Vesting Schedule may change prospectively in the event that Participant's Service status changes between full- and part-time and/or in the event the Participant is on a leave of absence, in accordance with Company policies relating to work schedules and vesting of Awards or as determined by the Committee. Furthermore, the period during which Participant may exercise the Option after termination of Service, if any, will commence on the Termination Date (as defined in the Option Agreement).

2) This grant is made under and governed by the Plan, the Agreement, and this Notice, and this Notice is subject to the terms and conditions of the Agreement and the Plan, both of which are incorporated herein by reference. Participant has read the Notice, the Option Agreement and, the Plan.

3) Participant has read the Company's Insider Trading Policy, and agrees to comply with such policy, as it may be amended from time to time, whenever Participant acquires or disposes of the Company's securities.

4) By accepting the Option, Participant consents to electronic delivery and participation as set forth in the Option Agreement.

PARTICIPANT

Signature: _____

Print Name: _____

STOKE THERAPEUTICS, INC.

By: _____

Its: _____

STOKE THERAPEUTICS, INC.
2019 EQUITY INCENTIVE PLAN
STOCK OPTION AWARD AGREEMENT

Unless otherwise defined in this Stock Option Award Agreement (this "***Option Agreement***"), any capitalized terms used herein will have the same meaning ascribed to them in the Stoke Therapeutics, Inc. 2019 Equity Incentive Plan (the "***Plan***").

Participant has been granted an option to purchase Shares (the "***Option***") of Stoke Therapeutics, Inc. (the "***Company***"), subject to the terms, restrictions, and conditions of the Plan, the Notice of Stock Option Grant (the "***Notice***"), and this Option Agreement, including any applicable country-specific provisions in any appendix attached hereto (the "***Appendix***"), which constitutes part of this Option Agreement. In the event of a conflict between the terms and conditions of the Plan and the terms and conditions of the Notice or this Option Agreement, the terms and conditions of the Plan will prevail.

1. Vesting Rights. Subject to the applicable provisions of the Plan and this Option Agreement, the Option may be exercised, in whole or in part, in accordance with the Vesting Schedule set forth in the Notice. Participant acknowledges and agrees that the Vesting Schedule may change prospectively in the event Participant's Service status changes between full and part-time and/or in the event Participant is on a leave of absence, in accordance with Company policies relating to work schedules and vesting of Awards or as determined by the Committee. Participant acknowledges that the vesting of the Option pursuant to this Notice and Agreement is subject to Participant's continuing Service as an Employee, Director, or Consultant.

2. Grant of Option. Participant has been granted an Option for the number of Shares set forth in the Notice at the exercise price per Share in U.S. Dollars set forth in the Notice (the "***Exercise Price***"). If designated in the Notice as an Incentive Stock Option ("***ISO***"), the Option is intended to qualify as an Incentive Stock Option under Section 422 of the Code. However, if the Option is intended to be an ISO, to the extent that it exceeds the U.S. $100,000 rule of Code Section 422(d) it will be treated as a Nonqualified Stock Option ("***NSO***").

3. Termination Period.

(a) General Rule. If Participant's Service terminates for any reason except death or Disability, and other than for Cause, then the Option will expire at the close of business at Company headquarters on the date three (3) months after Participant's Termination Date (as defined below) (or such shorter time period not less than thirty (30) days or longer time period as may be determined by the Committee, with any exercise beyond three (3) months after the date Participant's Service terminates deemed to be the exercise of an NSO). The Company determines when Participant's Service terminates for all purposes under this Option Agreement.

(b) Death; Disability. If Participant dies before Participant's Service terminates (or Participant dies within three (3) months of Participant's termination of Service other than for Cause), then the Option will expire at the close of business at Company headquarters on the date twelve (12) months after the date of death (or such shorter time period not less than six (6) months or longer time period as may be determined by the Committee, subject to the expiration details in Section 7). If Participant's Service terminates because of Participant's Disability, then the Option will expire at the close of business at Company headquarters on the date twelve (12) months after Participant's Termination Date (or such shorter time period not less than six (6) months or longer time period as may be determined by the Committee, subject to the expiration details in Section 7).

(c) Cause. Unless otherwise determined by the Committee, the Option (whether or not vested) will terminate immediately upon the Participant's cessation of Services if the Company reasonably determines in good faith that such cessation of Services has resulted in connection with an act

or failure to act constituting Cause (or the Participant's Services could have been terminated for Cause (without regard to the lapsing of any required notice or cure periods in connection therewith) at the time the Participant terminated Services).

(d) No Notification of Exercise Periods. Participant is responsible for keeping track of these exercise periods following Participant's termination of Service for any reason. The Company will not provide further notice of such periods. In no event will the Option be exercised later than the Expiration Date set forth in the Notice.

(e) Termination. For purposes of this Option, Participant's Service will be considered terminated as of the date Participant is no longer providing Services to the Company, its Parent or one of its Subsidiaries or Affiliates (regardless of the reason for such termination and whether or not later found to be invalid or in breach of employment laws in the jurisdiction where Participant is employed or the terms of Participant's employment agreement, if any) (the "***Termination Date***"). The Committee will have the exclusive discretion to determine when Participant is no longer actively providing services for purposes of Participant's Option (including whether Participant may still be considered to be providing services while on an approved leave of absence). Unless otherwise provided in this Option Agreement or determined by the Company, Participant's right to vest in this Option under the Plan, if any, will terminate as of the Termination Date and will not be extended by any notice period (*e.g.*, Participant's period of services would not include any contractual notice period or any period of "garden leave" or similar period mandated under employment laws in the jurisdiction where Participant is employed or the terms of Participant's employment agreement, if any). Following the Termination Date, Participant may exercise the Option only as set forth in the Notice and this Section, provided that the period (if any) during which Participant may exercise the Option after the Termination Date, if any, will commence on the date Participant ceases to provide services and will not be extended by any notice period mandated under employment laws in the jurisdiction where Participant is employed or terms of Participant's employment agreement, if any. If Participant does not exercise this Option within the termination period set forth in the Notice or the termination periods set forth above, the Option will terminate in its entirety. In no event, may any Option be exercised after the Expiration Date of the Option as set forth in the Notice.

4. Exercise of Option.

(a) Right to Exercise. The Option is exercisable during its term in accordance with the Vesting Schedule set forth in the Notice and the applicable provisions of the Plan and this Option Agreement. In the event of Participant's death, Disability, termination for Cause, or other cessation of Service, the exercisability of the Option is governed by the applicable provisions of the Plan, the Notice, and this Option Agreement. The Option may not be exercised for a fraction of a Share.

(b) Method of Exercise. The Option is exercisable by delivery of an exercise notice in a form specified by the Company (the "***Exercise Notice***"), which will state the election to exercise the Option, the number of Shares in respect of which the Option is being exercised (the "***Exercised Shares***"), and such other representations and agreements as may be required by the Company pursuant to the provisions of the Plan. The Exercise Notice will be delivered in person, by mail, via electronic mail or facsimile or by other authorized method to the Secretary of the Company or other person designated by the Company. The Exercise Notice will be accompanied by payment of the aggregate Exercise Price as to all Exercised Shares together with any applicable Tax-Related Items (as defined in Section 8 below). The Option will be deemed to be exercised upon receipt by the Company of such fully executed Exercise Notice accompanied by such aggregate Exercise Price and payment of any applicable Tax-Related Items. No Shares will be issued pursuant to the exercise of the Option unless such issuance and exercise complies with all relevant provisions of law and the requirements of any stock exchange or quotation service upon which the Shares are then listed. Assuming such compliance, for United States income tax purposes the Exercised Shares will be considered transferred to Participant on the date the Option is exercised with respect to such Exercised Shares.

(c) Exercise by Another. If another person wants to exercise the Option after it has been transferred to him or her in compliance with this Option Agreement, that person must prove to the Company's satisfaction that he or she is entitled to exercise the Option. That person must also complete the proper Exercise Notice form (as described above) and pay the Exercise Price (as described below) and any applicable Tax-Related Items (as described below).

5. Method of Payment. Payment of the aggregate Exercise Price will be by any of the following, or a combination thereof, at the election of Participant:

(a) Participant's personal check (or readily available funds), wire transfer, or a cashier's check;

(b) certificates for shares of Company stock that Participant owns, along with any forms needed to effect a transfer of those shares to the Company; the value of the shares, determined as of the effective date of the Option exercise, will be applied to the Exercise Price. Instead of surrendering shares of Company stock, Participant may attest to the ownership of those shares on a form provided by the Company and have the same number of shares subtracted from the Option shares issued to Participant. However, Participant may not surrender, or attest to the ownership of, shares of Company stock in payment of the Exercise Price of Participant's Option if Participant's action would cause the Company to recognize compensation expense (or additional compensation expense) with respect to this Option for financial reporting purposes;

(c) cashless exercise through irrevocable directions to a securities broker approved by the Company to sell all or part of the Shares covered by the Option and to deliver to the Company from the sale proceeds an amount sufficient to pay the Exercise Price and any applicable Tax-Related Items. The balance of the sale proceeds, if any, will be delivered to Participant. The directions must be given by signing a special notice of exercise form provided by the Company; or

(d) other method authorized by the Company;

provided, however, that the Company may restrict the available methods of payment due to facilitate compliance with applicable law or administration of the Plan. In particular, if Participant is located outside the United States, Participant should review the applicable provisions of the Appendix for any such restrictions that may currently apply.

6. Non-Transferability of Option. The Option may not be sold, assigned, transferred, pledged, hypothecated, or otherwise disposed of other than by will or by the laws of descent or distribution or court order and may be exercised during the lifetime of Participant only by Participant or unless otherwise permitted by the Committee on a case-by-case basis. The terms of the Plan and this Option Agreement will be binding upon the executors, administrators, heirs, successors, and assigns of Participant.

7. Term of Option. The Option will in any event expire on the expiration date set forth in the Notice, which date is ten (10) years after the Date of Grant (five (5) years after the Date of Grant if this option is designated as an ISO in the Notice of Stock Option Grant and Section 5.3 of the Plan applies).

8. Taxes.

(a) Responsibility for Taxes. Participant acknowledges that, regardless of any action taken by the Company or, if different, a Parent, Subsidiary, or Affiliate employing or retaining Participant (the "***Employer***"), the ultimate liability for all income tax, social insurance, payroll tax, fringe benefits tax, payment on account, or other tax related items related to Participant's participation in the Plan and legally applicable to Participant ("***Tax-Related Items***") is and remains Participant's responsibility and

may exceed the amount actually withheld by the Company or the Employer, if any. Participant further acknowledges that the Company and/or the Employer (i) make no representations or undertakings regarding the treatment of any Tax-Related Items in connection with any aspect of this Option, including, but not limited to, the grant, vesting, or exercise of this Option; the subsequent sale of Shares acquired pursuant to such exercise; and the receipt of any dividends; and (ii) do not commit to and are under no obligation to structure the terms of the grant or any aspect of this Option to reduce or eliminate Participant's liability for Tax-Related Items or achieve any particular tax result. Further, if Participant is subject to Tax-Related Items in more than one jurisdiction, Participant acknowledges that the Company and/or the Employer (or former employer, as applicable) may be required to withhold or account for Tax-Related Items in more than one jurisdiction. *PARTICIPANT SHOULD CONSULT A TAX ADVISER APPROPRIATELY QUALIFIED IN THE COUNTRY OR COUNTRIES IN WHICH PARTICIPANT RESIDES OR IS SUBJECT TO TAXATION.*

(b) Withholding. Prior to any relevant taxable or tax withholding event, as applicable, Participant agrees to make arrangements satisfactory to the Company and/or the Employer to satisfy all Tax-Related Items. In this regard, Participant authorizes the Company and/or the Employer, or their respective agents, at their discretion, to satisfy any withholding obligations for Tax-Related Items by one or a combination of the following, all under such rules as may be established by the Committee and in compliance with the Company's Insider Trading Policy and 10b5-1 Trading Plan Policy, if applicable:

(i) withholding from Participant's wages or other cash compensation paid to Participant by the Company and/or the Employer; or

(ii) withholding from proceeds of the sale of Shares acquired at exercise of this Option either through a voluntary sale or through a mandatory sale arranged by the Company (on Participant's behalf pursuant to this authorization and without further consent);

(iii) withholding Shares to be issued upon exercise of the Option, provided the Company only withholds the number of Shares necessary to satisfy no more than the maximum applicable statutory withholding amounts;

(iv) Participant's payment of a cash amount (including by check representing readily available funds or a wire transfer); or

(v) any other arrangement approved by the Committee and permitted under applicable law.

provided, however, that if Participant is a Section 16 officer of the Company under the Exchange Act, then the Committee (as constituted in accordance with Rule 16b-3 of the Exchange Act) shall establish the method of withholding from alternatives (i) – (v) above prior to the Tax-Related Items withholding event.

Depending on the withholding method, the Company may withhold or account for Tax-Related Items by considering applicable statutory withholding rates or other applicable withholding rates, including up to the maximum permissible statutory rate for Participant's tax jurisdiction(s) in which case Participant will have no entitlement to the equivalent amount in Shares and will receive a refund of any over-withheld amount in cash in accordance with applicable law. If the obligation for Tax-Related Items is satisfied by withholding in Shares, for tax purposes, Participant is deemed to have been issued the full number of Exercised Shares; notwithstanding that a number of the Shares are held back solely for the purpose of satisfying the withholding obligation for Tax-Related Items.

Finally, Participant agrees to pay to the Company and/or the Employer any amount of Tax-Related Items that the Company and/or the Employer may be required to withhold or account for as a result of Participant's participation in the Plan that cannot be satisfied by the means previously described. The Company may refuse to issue or deliver the Shares or the proceeds of the sale of Shares, if Participant fails to comply with Participant's obligations in connection with the Tax-Related Items.

(c) Notice of Disqualifying Disposition of ISO Shares. If Participant is subject to Tax-Related Items in the United States and sells or otherwise disposes of any of the Shares acquired pursuant to an ISO on or before the later of (i) two (2) years after the grant date, or (ii) one (1) year after the exercise date, Participant will immediately notify the Company in writing of such disposition. Participant agrees that he or she may be subject to income tax withholding by the Company on the compensation income recognized from such early disposition of ISO Shares by payment in cash or out any wages or other cash compensation paid to Participant by the Company and/or the Employer.

9. Nature of Grant. By accepting the Option, Participant acknowledges, understands and agrees that:

(a) the Plan is established voluntarily by the Company, it is discretionary in nature, and it may be modified, amended, suspended or terminated by the Company at any time, to the extent permitted by the Plan

(b) the grant of the Option is exceptional, voluntary, and occasional, and does not create any contractual or other right to receive future grants of options, or benefits in lieu of options, even if options have been granted in the past

(c) all decisions with respect to future options or other grants, if any, will be at the sole discretion of the Company;

(d) Participant is voluntarily participating in the Plan;

(e) the Option and Participant's participation in the Plan will not create a right to employment or be interpreted as forming or amending an employment or service contract with the Company or the Employer, and will not interfere with the ability of the Company or the Employer, as applicable, to terminate Participant's employment or service relationship (if any);

(f) the Option and the Shares subject to the Option, and the income and value of same, are not intended to replace any pension rights or compensation;

(g) the Option and the Shares subject to the Option, and the income and value of same, are not part of normal or expected compensation for any purpose, including, but not limited to, calculating any severance, resignation, termination, redundancy, dismissal, end-of-service payments, bonuses, long-service awards, pension or retirement, or welfare benefits or similar payments;

(h) unless otherwise agreed with the Company, the Option, and the Shares subject to the Option, and the income and value of same, are not granted as consideration for, or in connection with, the service Participant may provide as a director of a Parent, Subsidiary, or Affiliate;

(i) the future value of the Shares underlying the Option is unknown, indeterminable, and cannot be predicted with certainty; if the underlying Shares do not increase in value, the Option will have no value; if Participant exercises the Option and acquires Shares, the value of such Shares may increase or decrease, even below the Exercise Price;

(j) no claim or entitlement to compensation or damages will arise from forfeiture of the Option resulting from Participant's termination of Service (regardless of the reason for such termination and whether or not later found to be invalid or in breach of employment laws in the jurisdiction where Participant is employed or the terms of Participant's employment agreement, if any), and in consideration of the grant of the Option to which Participant is otherwise not entitled, Participant irrevocably agrees never to institute any claim against the Employer, the Company, and any Parent,

Subsidiary, or Affiliate; waives his or her ability, if any, to bring any such claim; and releases the Employer, the Company, and any Parent, Subsidiary, or Affiliate from any such claim; if, notwithstanding the foregoing, any such claim is allowed by a court of competent jurisdiction, then, by participating in the Plan, Participant will be deemed irrevocably to have agreed not to pursue such claim and agrees to execute any and all documents necessary to request dismissal or withdrawal of such claim;

(k) unless otherwise provided in the Plan or by the Company in its discretion, the Option and the benefits evidenced by this Option Agreement do not create any entitlement to have the Option or any such benefits transferred to, or assumed by, another company nor to be exchanged, cashed out or substituted for, in connection with any Corporate Transaction affecting the Shares; an

(l) neither the Employer, the Company, or any Parent, Subsidiary or Affiliate will be liable for any foreign exchange rate fluctuation between Participant's local currency and the United States Dollar that may affect the value of the Option or of any amounts due to Participant pursuant to the exercise of the Option or the subsequent sale of any Shares acquired upon exercise

(m) the following provisions apply only if Participant is providing services outside the United States:

(i) the Option and the Shares subject to the Option are not part of normal or expected compensation or salary for any purpose; and

(ii) Participant acknowledges and agrees that neither the Company, the Employer nor any Parent or Subsidiary or Affiliate will be liable for any foreign exchange rate fluctuation between Participant's local currency and the United States Dollar that may affect the value of the Option or of any amounts due to Participant pursuant to the exercise of the Option or the subsequent sale of any Shares acquired upon exercised.

10. No Advice Regarding Grant. The Company is not providing any tax, legal or financial advice, nor is the Company making any recommendations regarding Participant's participation in the Plan or Participant's acquisition or sale of the underlying Shares. Participant acknowledges, understands, and agrees that he or she should consult with his or her own personal tax, legal, and financial advisors regarding his or her participation in the Plan before taking any action related to the Plan.

11. Data Privacy. ***Participant hereby explicitly and unambiguously consents to the collection, use and transfer, in electronic or other form, of Participant's personal data as described in this Option Agreement and any other Option grant materials by and among, as applicable, the Employer, the Company and any Parent, Subsidiary or Affiliate for the exclusive purpose of implementing, administering and managing Participant's participation in the Plan.***

Participant understands that the Company and the Employer may hold certain personal information about Participant, including, but not limited to, Participant's name, home address, email address and telephone number, date of birth, social insurance number, passport number or other identification number, salary, nationality, job title, any shares of stock or directorships held in the Company, details of all Options or any other entitlement to shares of stock awarded, canceled, exercised, vested, unvested or outstanding in Participant's favor ("Data"), for the exclusive purpose of implementing, administering and managing the Plan.

Participant understands that Data will be transferred to the stock plan service provider as may be designated by the Company from time to time or its affiliates or such other stock plan service provider as may be selected by the Company in the future, which is assisting the Company with the implementation, administration and management of the Plan. Participant understands that the recipients of the Data may be located in the United States or elsewhere, and that the recipients' country

(e.g., the United States) may have different data privacy laws and protections than Participant's country. Participant understands that if he or she resides outside the United States, he or she may request a list with the names and addresses of any potential recipients of the Data by contacting his or her local human resources representative. Participant authorizes the Company, the stock plan service provider as may be designated by the Company from time to time, and its affiliates, and any other possible recipients which may assist the Company (presently or in the future) with implementing, administering and managing the Plan to receive, possess, use, retain and transfer the Data, in electronic or other form, for the sole purpose of implementing, administering and managing his or her participation in the Plan. Participant understands that Data will be held only as long as is necessary to implement, administer and manage Participant's participation in the Plan. Participant understands that if he or she resides outside the United States, he or she may, at any time, view Data, request additional information about the storage and processing of Data, require any necessary amendments to Data or refuse or withdraw the consents herein, in any case without cost, by contacting in writing his or her local human resources representative. Further, Participant understands that he or she is providing the consents herein on a purely voluntary basis. If Participant does not consent, or if Participant later seeks to revoke his or her consent, his or her employment status or service and career with the Employer will not be adversely affected; the only adverse consequence of refusing or withdrawing Participant's consent is that the Company would not be able to grant Participant options or other equity awards or administer or maintain such awards. Therefore, Participant understands that refusing or withdrawing his or her consent may affect Participant's ability to participate in the Plan. For more information on the consequences of Participant's refusal to consent or withdrawal of consent, Participant understands that he or she may contact his or her local human resources representative.

12. Language. If Participant has received this Option Agreement, or any other document related to the Option and/or the Plan translated into a language other than English and if the meaning of the translated version is different than the English version, the English version will control.

13. Appendix. Notwithstanding any provisions in this Option Agreement, the Option will be subject to any special terms and conditions set forth in any Appendix to this Option Agreement for Participant's country. Moreover, if Participant relocates to one of the countries included in the Appendix, the special terms and conditions for such country will apply to Participant, to the extent the Company determines that the application of such terms and conditions is necessary or advisable for legal or administrative reasons. The Appendix constitutes part of this Option Agreement.

14. Imposition of Other Requirements. The Company reserves the right to impose other requirements on Participant's participation in the Plan, on the Option, and on any Shares purchased upon exercise of the Option, to the extent the Company determines it is necessary or advisable for legal or administrative reasons, and to require Participant to sign any additional agreements or undertakings that may be necessary to accomplish the foregoing.

15. Acknowledgement. The Company and Participant agree that the Option is granted under and governed by the Notice, this Option Agreement and the Plan (incorporated herein by reference). Participant: (a) acknowledges receipt of a copy of the Plan and the Plan prospectus, (b) represents that Participant has carefully read and is familiar with their provisions, and (c) hereby accepts the Option subject to all of the terms and conditions set forth herein and those set forth in the Plan and the Notice.

16. Entire Agreement; Enforcement of Rights. This Option Agreement, the Plan, and the Notice constitute the entire agreement and understanding of the parties relating to the subject matter herein and supersede all prior discussions between them. Any prior agreements, commitments, or negotiations concerning the purchase of the Shares hereunder are superseded. No adverse modification of, or adverse amendment to, this Option Agreement, nor any waiver of any rights under this Option Agreement, will be effective unless in writing and signed by the parties to this Option Agreement (which writing and signing may be electronic). The failure by either party to enforce any rights under this Option Agreement will not be construed as a waiver of any rights of such party.

17. Compliance with Laws and Regulations. The issuance of Shares and the sale of Shares will be subject to and conditioned upon compliance by the Company and Participant with all applicable state, federal, local and foreign laws and regulations and with all applicable requirements of any stock exchange or automated quotation system on which the Company's Shares may be listed or quoted at the time of such issuance or transfer. Participant understands that the Company is under no obligation to register or qualify the Common Stock with any state, federal, or foreign securities commission or to seek approval or clearance from any governmental authority for the issuance or sale of the Shares. Further, Participant agrees that the Company will have unilateral authority to amend the Plan and this Option Agreement without Participant's consent to the extent necessary to comply with securities or other laws applicable to issuance of Shares. Finally, the Shares issued pursuant to this Option Agreement will be endorsed with appropriate legends, if any, determined by the Company.

18. Severability. If one or more provisions of this Option Agreement are held to be unenforceable under applicable law, the parties agree to renegotiate such provision in good faith. In the event that the parties cannot reach a mutually agreeable and enforceable replacement for such provision, then (a) such provision will be excluded from this Option Agreement, (b) the balance of this Option Agreement will be interpreted as if such provision were so excluded and (c) the balance of this Option Agreement will be enforceable in accordance with its terms.

19. Governing Law and Venue. This Option Agreement and all acts and transactions pursuant hereto and the rights and obligations of the parties hereto will be governed, construed and interpreted in accordance with the laws of the State of Delaware, without giving effect to such state's conflict of laws rules.

Any and all disputes relating to, concerning or arising from this Option Agreement, or relating to, concerning or arising from the relationship between the parties evidenced by the Plan or this Option Agreement, will be brought and heard exclusively in the U.S. District Court for the District of Massachusetts or the Massachusetts Superior Court, Middlesex County. Each of the parties hereby represents and agrees that such party is subject to the personal jurisdiction of said courts; hereby irrevocably consents to the jurisdiction of such courts in any legal or equitable proceedings related to, concerning, or arising from such dispute, and waives, to the fullest extent permitted by law, any objection which such party may now or hereafter have that the laying of the venue of any legal or equitable proceedings related to, concerning, or arising from such dispute which is brought in such courts is improper or that such proceedings have been brought in an inconvenient forum.

20. No Rights as Employee, Director or Consultant. Nothing in this Option Agreement will affect in any manner whatsoever any right or power of the Employer or the Company to terminate Participant's Service, for any reason, with or without Cause.

21. Consent to Electronic Delivery of All Plan Documents and Disclosures. By Participant's acceptance of the Notice (whether in writing or electronically), Participant and the Company agree that the Option is granted under and governed by the terms and conditions of the Plan, the Notice, and this Option Agreement. Participant has reviewed the Plan, the Notice, and this Option Agreement in their entirety, has had an opportunity to obtain the advice of counsel prior to executing the Notice and Agreement, and fully understands all provisions of the Plan, the Notice, and this Option Agreement. Participant hereby agrees to accept as binding, conclusive, and final all decisions or interpretations of the Committee upon any questions relating to the Plan, the Notice, and this Option Agreement. Participant further agrees to notify the Company upon any change in Participant's residence address. By acceptance of the Option, Participant agrees to participate in the Plan through an on-line or electronic system

established and maintained by the Company or a third party designated by the Company and consents to the electronic delivery of the Notice, this Option Agreement, the Plan, account statements, Plan prospectuses required by the U.S. Securities and Exchange Commission, U.S. financial reports of the Company, and all other documents that the Company is required to deliver to its security holders (including, without limitation, annual reports and proxy statements), or other communications or information related to the Option and current or future participation in the Plan. Electronic delivery may include the delivery of a link to the Company intranet or the internet site of a third party involved in administering the Plan, the delivery of the document via e-mail, or such other delivery determined at the Company's discretion. Participant acknowledges that Participant may receive from the Company a paper copy of any documents delivered electronically at no cost if Participant contacts the Company by telephone, through a postal service, or electronic mail to Stock Administration. Participant further acknowledges that Participant will be provided with a paper copy of any documents delivered electronically if electronic delivery fails; similarly, Participant understands that Participant must provide on request to the Company or any designated third party a paper copy of any documents delivered electronically if electronic delivery fails. Also, Participant understands that Participant's consent may be revoked or changed, including any change in the electronic mail address to which documents are delivered (if Participant has provided an electronic mail address), at any time by notifying the Company of such revised or revoked consent by telephone, postal service, or electronic mail to Stock Administration. Finally, Participant understands that Participant is not required to consent to electronic delivery if local laws prohibit such consent.

22. Insider Trading Restrictions/Market Abuse Laws. Participant acknowledges that, depending on Participant's country, Participant may be subject to insider trading restrictions and/or market abuse laws, which may affect Participant's ability to acquire or sell the Shares or rights to Shares under the Plan during such times as Participant is considered to have "inside information" regarding the Company (as defined by the laws in Participant's country). Any restrictions under these laws or regulations are separate from and in addition to any restrictions that may be imposed under any applicable Company insider trading policy. Participant acknowledges that it is Participant's responsibility to comply with any applicable restrictions and understands that Participant should consult his or her personal legal advisor on such matters. In addition, Participant acknowledges that he or she has read the Company's Insider Trading Policy, and agrees to comply with such policy, as it may be amended from time to time, whenever Participant acquires or disposes of the Company's securities.

23. Award Subject to Company Clawback or Recoupment. To the extent permitted by applicable law, the Option will be subject to clawback or recoupment pursuant to any compensation clawback or recoupment policy adopted by the Board or required by law during the term of Participant's employment or other Service that is applicable to Participant. In addition to any other remedies available under such policy and applicable law, the Company may require the cancellation of Participant's Option (whether vested or unvested) and the recoupment of any gains realized with respect to Participant's Option.

BY ACCEPTING THIS OPTION, PARTICIPANT AGREES TO ALL OF THE TERMS AND CONDITIONS DESCRIBED ABOVE AND IN THE PLAN.

APPENDIX
STOKE THERAPEUTICS, INC.
2019 EQUITY INCENTIVE PLAN
STOCK OPTION AWARD AGREEMENT
COUNTRY SPECIFIC PROVISIONS FOR EMPLOYEES OUTSIDE THE U.S.

Terms and Conditions

This Appendix includes additional terms and conditions that govern the Option granted to Participant under the Plan if Participant resides and/or works in one of the countries below. This Appendix forms part of the Option Agreement. Any capitalized term used in this Appendix without definition will have the meaning ascribed to it in the Notice, the Option Agreement, or the Plan, as applicable.

If Participant is a citizen or resident of a country, or is considered resident of a country, other than the one in which Participant is currently working, or Participant transfers employment and/or residency between countries after the Date of Grant, the Company will, in its sole discretion, determine to what extent the additional terms and conditions included herein will apply to Participant under these circumstances.

Notifications

This Appendix also includes information relating to exchange control, securities laws, foreign asset/account reporting, and other issues of which Participant should be aware with respect to Participant's participation in the Plan. The information is based on the securities, exchange control, foreign asset/account reporting, and other laws in effect in the respective countries as of June 4, 2019. Such laws are complex and change frequently. As a result, Participant should not rely on the information herein as the only source of information relating to the consequences of Participant's participation in the Plan because the information may be out of date at the time that Participant exercises the Option, sells Shares acquired under the Plan, or takes any other action in connection with the Plan.

In addition, the information is general in nature and may not apply to Participant's particular situation, and the Company is not in a position to assure Participant of any particular result. Accordingly, Participant should seek appropriate professional advice as to how the relevant laws in Participant's country may apply to Participant's situation.

Finally, if Participant is a citizen or resident of a country, or is considered resident of a country, other than the one in which Participant is currently working and/or residing, or Participant transfers employment and/or residency after the Date of Grant, the information contained herein may not apply to Participant in the same manner.

APPENDIX

STOKE THERAPEUTICS, INC.
2019 EQUITY INCENTIVE PLAN
STOCK OPTION AWARD AGREEMENT

COUNTRY SPECIFIC PROVISIONS FOR EMPLOYEES OUTSIDE THE U.S.

None

STOKE THERAPEUTICS, INC.
2019 EQUITY INCENTIVE PLAN
NOTICE OF RESTRICTED STOCK UNIT AWARD

Unless otherwise defined herein, the terms defined in the Stoke Therapeutics, Inc. (the "***Company***") 2019 Equity Incentive Plan (the "***Plan***") will have the same meanings in this Notice of Restricted Stock Unit Award and the electronic representation of this Notice of Restricted Stock Unit Award established and maintained by the Company or a third party designated by the Company (this "***Notice***").

Name:

Address:

You (the "***Participant***") have been granted an award of Restricted Stock Units ("***RSUs***") under the Plan subject to the terms and conditions of the Plan, this Notice and the attached Restricted Stock Unit Award Agreement (the "***Agreement***"), including any applicable country-specific provisions in any appendix attached hereto (the "***Appendix***"), which constitutes part of the Agreement.

Grant Number:

Number of RSUs:

Date of Grant:

Vesting Commencement Date:

Expiration Date:	The earlier to occur of: (a) the date on which settlement of all RSUs granted hereunder occurs, and (b) the tenth anniversary of the Date of Grant. This RSU expires earlier if Participant's Service terminates earlier, as described in the Agreement.
Vesting Schedule:	Subject to the limitations set forth in this Notice, the Plan, and the Agreement, the RSUs will vest in accordance with the following schedule: [*insert applicable vesting schedule*]

By accepting (whether in writing, electronically or otherwise) the RSUs, Participant acknowledges and agrees to the following:

1) Participant understands that Participant's Service with the Company or a Parent, Subsidiary, or Affiliate is for an unspecified duration, can be terminated at any time (*i.e.*, is "at-will"), except where otherwise prohibited by applicable law, and that nothing in this Notice, the Agreement, or the Plan changes the nature of that relationship. Participant acknowledges that the vesting of the RSUs pursuant to this Notice is subject to Participant's continuing Service as an Employee, Director or Consultant. Participant agrees and acknowledges that the Vesting Schedule may change prospectively in the event that Participant's Service status changes between full- and part-time and/or in the event the Participant is on a leave of absence, in accordance with Company policies relating to work schedules and vesting of Awards or as determined by the Committee.

2) This grant is made under and governed by the Plan, the Agreement, and this Notice, and this Notice is subject to the terms and conditions of the Agreement and the Plan, both of which are incorporated herein by reference. Participant has read the Notice, the Agreement, and the Plan.

3) Participant has read the Company's Insider Trading Policy, and agrees to comply with such policy, as it may be amended from time to time, whenever Participant acquires or disposes of the Company's securities.

4) By accepting the RSUs, Participant consents to electronic delivery and participation as set forth in the Agreement.

PARTICIPANT		**STOKE THERAPEUTICS, INC.**	
Signature:	_____	By:	_____
Print Name:	_____	Its:	_____

STOKE THERAPEUTICS, INC.
2019 EQUITY INCENTIVE PLAN
RESTRICTED STOCK UNIT AWARD AGREEMENT

Unless otherwise defined in this Restricted Stock Unit Award Agreement (this "***Agreement***"), any capitalized terms used herein will have the same meaning ascribed to them in the Stoke Therapeutics, Inc. 2019 Equity Incentive Plan (the "***Plan***").

Participant has been granted Restricted Stock Units ("***RSUs***") subject to the terms, restrictions, and conditions of the Plan, the Notice of Restricted Stock Unit Award (the "***Notice***"), and this Agreement, including any applicable country-specific provisions in any appendix attached hereto (the "***Appendix***"), which constitutes part of this Agreement. In the event of a conflict between the terms and conditions of the Plan and the terms and conditions of the Notice or this Agreement, the terms and conditions of the Plan will prevail.

1. Settlement. Settlement of RSUs will be made within thirty (30) days following the applicable date of vesting under the Vesting Schedule set forth in the Notice. Settlement of RSUs will be in Shares. No fractional RSUs or rights for fractional Shares will be created pursuant to this Agreement.

2. No Stockholder Rights. Unless and until such time as Shares are issued in settlement of vested RSUs, Participant will have no ownership of the Shares allocated to the RSUs and will have no rights to dividends or to vote such Shares.

3. Dividend Equivalents. Dividends, if any (whether in cash or Shares), will not be credited to Participant.

4. Non-Transferability of RSUs. The RSUs and any interest therein will not be sold, assigned, transferred, pledged, hypothecated, or otherwise disposed of in any manner other than by will or by the laws of descent or distribution or court order or unless otherwise permitted by the Committee on a case-by-case basis.

5. Termination; Leave of Absence; Change in Status. If Participant's Service terminates for any reason, all unvested RSUs will be forfeited to the Company immediately, and all rights of Participant to such RSUs automatically terminate without payment of any consideration to Participant. Participant's Service will be considered terminated as of the date Participant is no longer providing services (regardless of the reason for such termination and whether or not later found to be invalid or in breach of employment laws in the jurisdiction where Participant is employed or the terms of Participant's employment agreement, if any) and will not, subject to the laws applicable to Participant's Award, be extended by any notice period mandated under local laws (e.g., Service would not include a period of "garden leave" or similar period). Participant acknowledges and agrees that the Vesting Schedule may change prospectively in the event Participant's service status changes between full- and part-time status and/or in the event Participant is on an approved leave of absence in accordance the Company's policies relating to work schedules and vesting of awards or as determined by the Committee. Participant acknowledges that the vesting of the Shares pursuant to this Notice and Agreement is subject to Participant's continued Service. In case of any dispute as to whether termination of Service has occurred, the Committee will have sole discretion to determine whether such termination of Service has occurred and the effective date of such termination (including whether Participant may still be considered to be providing services while on an approved leave of absence).

6. Taxes.

(a) Responsibility for Taxes. Participant acknowledges that, regardless of any action taken by the Company or, if different, a Parent, Subsidiary or Affiliate employing or retaining Participant (the "***Employer***"), the ultimate liability for all income tax, social insurance, payroll tax, fringe benefits tax, payment on account or other tax-related items related to Participant's participation in the Plan and legally applicable to Participant ("***Tax-Related Items***") is and remains Participant's responsibility and may exceed the amount actually withheld by the Company or the Employer, if any. Participant further acknowledges that the Company and/or the Employer (i) make no representations or undertakings regarding the treatment of any Tax-Related Items in connection with any aspect of the RSUs, including, but not limited to, the grant, vesting or settlement of the RSUs and the subsequent sale of Shares acquired pursuant to such settlement and the receipt of any dividends, and (ii) do not commit to and are under no obligation to structure the terms of the grant or any aspect of the RSUs to reduce or eliminate Participant's liability for Tax-Related Items or achieve any particular tax result. Further, if Participant is subject to Tax-Related Items in more than one jurisdiction, Participant acknowledges that the Company and/or the Employer (or former employer, as applicable) may be required to withhold or account for Tax-Related Items in more than one jurisdiction. *PARTICIPANT SHOULD CONSULT A TAX ADVISER APPROPRIATELY QUALIFIED IN THE COUNTRY OR COUNTRIES IN WHICH PARTICIPANT RESIDES OR IS SUBJECT TO TAXATION.*

(b) Withholding. Prior to any relevant taxable or tax withholding event, as applicable, Participant agrees to make arrangements satisfactory to the Company and/or the Employer to satisfy all Tax-Related Items. In this regard, Participant authorizes the Company and/or the Employer, or their respective agents, at their discretion, to satisfy any withholding obligations for Tax-Related Items by one or a combination of the following all under such rules as may be established by the Committee and in compliance with the Company's Insider Trading Policy and 10b5-1 Trading Plan Policy, if applicable:

(i) withholding from Participant's wages or other cash compensation paid to Participant by the Company and/or the Employer; or

(ii) withholding from proceeds of the sale of Shares acquired upon settlement of the RSUs either through a voluntary sale or through a mandatory sale arranged by the Company (on Participant's behalf pursuant to this authorization and without further consent);

(iii) withholding Shares to be issued upon settlement of the RSUs, provided the Company only withholds the number of Shares necessary to satisfy no more than the maximum applicable statutory withholding amounts;

(iv) Participant's payment of a cash amount (including by check representing readily available funds or a wire transfer); or

(v) any other arrangement approved by the Committee and permitted under applicable law;

provided however, that if Participant is a Section 16 officer of the Company under the Exchange Act, then the Committee (as constituted in accordance with Rule 16b-3 under the Exchange Act) will establish the method of withholding from alternatives (i)-(v) above prior to the Tax-Related Items withholding event.

Depending on the withholding method, the Company may withhold or account for Tax-Related Items by considering applicable statutory withholding rates or other applicable withholding rates, including up to the maximum permissible statutory rate for Participant's tax jurisdiction(s) in which case Participant will have no entitlement to the equivalent amount in Shares and will receive a refund of any over-withheld

amount in cash in accordance with applicable law. If the obligation for Tax-Related Items is satisfied by withholding in Shares, for tax purposes, Participant is deemed to have been issued the full number of Shares subject to the vested RSUs, notwithstanding that a number of the Shares are held back solely for the purpose of satisfying the withholding obligation for Tax-Related Items.

Finally, Participant agrees to pay to the Company and/or the Employer any amount of Tax-Related Items that the Company and/or the Employer may be required to withhold or account for as a result of Participant's participation in the Plan that cannot be satisfied by the means previously described. The Company may refuse to issue or deliver the Shares or the proceeds of the sale of Shares, if Participant fails to comply with Participant's obligations in connection with the Tax-Related Items.

7. Nature of Grant. By accepting the RSUs, Participant acknowledges, understands and agrees that:

(a) the Plan is established voluntarily by the Company, it is discretionary in nature and it may be modified, amended, suspended or terminated by the Company at any time, to the extent permitted by the Plan;

(b) the grant of the RSUs is exceptional, voluntary, and occasional, and does not create any contractual or other right to receive future grants of RSUs, or benefits in lieu of RSUs, even if RSUs have been granted in the past;

(c) all decisions with respect to future RSUs or other grants, if any, will be at the sole discretion of the Company;

(d) Participant is voluntarily participating in the Plan;

(e) the RSUs and Participant's participation in the Plan will not create a right to employment or be interpreted as forming or amending an employment or service contract with the Company or the Employer and will not interfere with the ability of the Company or the Employer, as applicable, to terminate Participant's employment or service relationship (if any);

(f) the RSUs and the Shares subject to the RSUs, and the income and value of same, are not intended to replace any pension rights or compensation;

(g) the RSUs and the Shares subject to the RSUs, and the income and value of same, are not part of normal or expected compensation for any purpose, including, but not limited to, calculating any severance, resignation, termination, redundancy, dismissal, end-of-service payments, bonuses, long-service awards, pension or retirement, or welfare benefits or similar payments;

(h) unless otherwise agreed with the Company, the RSUs, and the Shares subject to the RSUs, and the income and value of same, are not granted as consideration for, or in connection with, the service Participant may provide as a director of a Parent, Subsidiary, or Affiliate;

(i) the future value of the underlying Shares is unknown, indeterminable, and cannot be predicted with certainty;

(j) no claim or entitlement to compensation or damages will arise from forfeiture of the RSUs resulting from Participant's termination of Service (regardless of the reason for such termination and whether or not later found to be invalid or in breach of employment laws in the jurisdiction where Participant is employed or the terms of Participant's employment agreement, if any), and in consideration of the grant of the RSUs to which Participant is otherwise not entitled, Participant irrevocably agrees never

to institute any claim against the Employer, the Company, and any Parent, Subsidiary or Affiliate; waives his or her ability, if any, to bring any such claim; and releases the Employer, the Company, and any Parent, Subsidiary, or Affiliate from any such claim; if, notwithstanding the foregoing, any such claim is allowed by a court of competent jurisdiction, then, by participating in the Plan, Participant will be deemed irrevocably to have agreed not to pursue such claim and agrees to execute any and all documents necessary to request dismissal or withdrawal of such claim;

(k) unless otherwise provided in the Plan or by the Company in its discretion, the RSUs and the benefits evidenced by this Agreement do not create any entitlement to have the RSUs or any such benefits transferred to, or assumed by, another company nor to be exchanged, cashed out or substituted for, in connection with any Corporate Transaction affecting the Shares; and

(l) the following provisions apply only if Participant is providing services outside the United States:

(i) the RSUs and the Shares subject to the RSUs are not part of normal or expected compensation or salary for any purpose;

(ii) Participant acknowledges and agrees that neither the Company, the Employer nor any Parent or Subsidiary or Affiliate will be liable for any foreign exchange rate fluctuation between Participant's local currency and the United States Dollar that may affect the value of the RSUs or of any amounts due to Participant pursuant to the settlement of the RSUs or the subsequent sale of any Shares acquired upon settlement.

8. No Advice Regarding Grant. The Company is not providing any tax, legal, or financial advice, nor is the Company making any recommendations regarding Participant's participation in the Plan, or Participant's acquisition or sale of the underlying Shares. Participant acknowledges, understands and agrees he or she should consult with his or her own personal tax, legal, and financial advisors regarding his or her participation in the Plan before taking any action related to the Plan.

9. Data Privacy. ***Participant hereby explicitly and unambiguously consents to the collection, use and transfer, in electronic or other form, of Participant's personal data as described in this Agreement and any other RSU grant materials by and among, as applicable, the Employer, the Company and any Parent, Subsidiary or Affiliate for the exclusive purpose of implementing, administering and managing Participant's participation in the Plan.***

Participant understands that the Company and the Employer may hold certain personal information about Participant, including, but not limited to, Participant's name, home address, email address and telephone number, date of birth, social insurance number, passport number or other identification number, salary, nationality, job title, any shares of stock or directorships held in the Company, details of all RSUs or any other entitlement to shares of stock awarded, canceled, exercised, vested, unvested or outstanding in Participant's favor ("Data"), for the exclusive purpose of implementing, administering and managing the Plan.

Participant understands that Data will be transferred to the stock plan service provider as may be designated by the Company from time to time or its affiliates or such other stock plan service provider as may be selected by the Company in the future, which is assisting the Company with the implementation, administration and management of the Plan. Participant understands that the recipients of the Data may be located in the United States or elsewhere, and that the recipients' country (e.g., the United States) may have different data privacy laws and protections than Participant's country. Participant understands that if he or she resides outside the United States, he or she may request a list

with the names and addresses of any potential recipients of the Data by contacting his or her local human resources representative. Participant authorizes the Company, the stock plan service provider as may be designated by the Company from time to time, and its affiliates, and any other possible recipients which may assist the Company (presently or in the future) with implementing, administering and managing the Plan to receive, possess, use, retain and transfer the Data, in electronic or other form, for the sole purpose of implementing, administering and managing his or her participation in the Plan. Participant understands that Data will be held only as long as is necessary to implement, administer and manage Participant's participation in the Plan. Participant understands that if he or she resides outside the United States, he or she may, at any time, view Data, request additional information about the storage and processing of Data, require any necessary amendments to Data or refuse or withdraw the consents herein, in any case without cost, by contacting in writing his or her local human resources representative. Further, Participant understands that he or she is providing the consents herein on a purely voluntary basis. If Participant does not consent, or if Participant later seeks to revoke his or her consent, his or her employment status or service and career with the Employer will not be adversely affected; the only adverse consequence of refusing or withdrawing Participant's consent is that the Company would not be able to grant Participant RSUs or other equity awards or administer or maintain such awards. Therefore, Participant understands that refusing or withdrawing his or her consent may affect Participant's ability to participate in the Plan. For more information on the consequences of Participant's refusal to consent or withdrawal of consent, Participant understands that he or she may contact his or her local human resources representative.

10. Language. If Participant has received this Agreement or any other document related to the RSU and/or the Plan translated into a language other than English and if the meaning of the translated version is different than the English version, the English version will control.

11. Appendix. Notwithstanding any provisions in this Agreement, the RSUs will be subject to any special terms and conditions set forth in any Appendix to this Agreement for Participant's country. Moreover, if Participant relocates to one of the countries included in the Appendix, the special terms and conditions for such country will apply to Participant, to the extent the Company determines that the application of such terms and conditions is necessary or advisable for legal or administrative reasons. The Appendix constitutes part of this Agreement.

12. Imposition of Other Requirements. The Company reserves the right to impose other requirements on Participant's participation in the Plan, on the RSUs and on any Shares acquired under the Plan, to the extent the Company determines it is necessary or advisable for legal or administrative reasons, and to require Participant to sign any additional agreements or undertakings that may be necessary to accomplish the foregoing.

13. Acknowledgement. The Company and Participant agree that the RSUs are granted under and governed by the Notice, this Agreement, and the Plan (incorporated herein by reference). Participant: (a) acknowledges receipt of a copy of the Plan and the Plan prospectus, (b) represents that Participant has carefully read and is familiar with their provisions, and (c) hereby accepts the RSUs subject to all of the terms and conditions set forth herein and those set forth in the Plan and the Notice.

14. Entire Agreement; Enforcement of Rights. This Agreement, the Plan, and the Notice constitute the entire agreement and understanding of the parties relating to the subject matter herein and supersede all prior discussions between them. Any prior agreements, commitments, or negotiations concerning the purchase of the Shares hereunder are superseded. No adverse modification of or adverse amendment to this Agreement, nor any waiver of any rights under this Agreement, will be effective unless in writing and signed by the parties to this Agreement (which writing and signing may be electronic). The failure by either party to enforce any rights under this Agreement will not be construed as a waiver of any rights of such party.

15. Compliance with Laws and Regulations. The issuance of Shares and the sale of Shares will be subject to and conditioned upon compliance by the Company and Participant with all applicable state, federal, local and foreign laws and regulations and with all applicable requirements of any stock exchange or automated quotation system on which the Company's Shares may be listed or quoted at the time of such issuance or transfer. Participant understands that the Company is under no obligation to register or qualify the Common Stock with any state, federal, or foreign securities commission or to seek approval or clearance from any governmental authority for the issuance or sale of the Shares. Further, Participant agrees that the Company will have unilateral authority to amend the Plan and this RSU Agreement without Participant's consent to the extent necessary to comply with securities or other laws applicable to issuance of Shares. Finally, the Shares issued pursuant to this RSU Agreement will be endorsed with appropriate legends, if any, determined by the Company.

16. Severability. If one or more provisions of this Agreement are held to be unenforceable under applicable law, the parties agree to renegotiate such provision in good faith. In the event that the parties cannot reach a mutually agreeable and enforceable replacement for such provision, then (a) such provision will be excluded from this Agreement, (b) the balance of this Agreement will be interpreted as if such provision were so excluded and (c) the balance of this Agreement will be enforceable in accordance with its terms.

17. Governing Law and Venue. This Agreement and all acts and transactions pursuant hereto and the rights and obligations of the parties hereto will be governed, construed, and interpreted in accordance with the laws of the State of Delaware, without giving effect to such state's conflict of laws rules.

Any and all disputes relating to, concerning or arising from this Agreement, or relating to, concerning, or arising from the relationship between the parties evidenced by the Plan or this Agreement, will be brought and heard exclusively in the U.S. District Court for the District of Massachusetts or the Massachusetts Superior Court, Middlesex County. Each of the parties hereby represents and agrees that such party is subject to the personal jurisdiction of said courts; hereby irrevocably consents to the jurisdiction of such courts in any legal or equitable proceedings related to, concerning, or arising from such dispute, and waives, to the fullest extent permitted by law, any objection which such party may now or hereafter have that the laying of the venue of any legal or equitable proceedings related to, concerning, or arising from such dispute which is brought in such courts is improper or that such proceedings have been brought in an inconvenient forum.

18. No Rights as Employee, Director or Consultant. Nothing in this Agreement will affect in any manner whatsoever any right or power of the Employer or the Company to terminate Participant's Service, for any reason, with or without Cause.

19. Consent to Electronic Delivery of All Plan Documents and Disclosures. By Participant's acceptance of the Notice (whether in writing or electronically), Participant and the Company agree that the RSUs are granted under and governed by the terms and conditions of the Plan, the Notice, and this Agreement. Participant has reviewed the Plan, the Notice, and this Agreement in their entirety, has had an opportunity to obtain the advice of counsel prior to executing this Notice and Agreement, and fully understands all provisions of the Plan, the Notice, and this Agreement. Participant hereby agrees to accept as binding, conclusive, and final all decisions or interpretations of the Committee upon any questions relating to the Plan, the Notice, and this Agreement. Participant further agrees to notify the Company upon any change in Participant's residence address. By acceptance of the RSUs, Participant agrees to participate in the Plan through an on-line or electronic system established and maintained by the Company or a third

party designated by the Company and consents to the electronic delivery of the Notice, this Agreement, the Plan, account statements, Plan prospectuses required by the U.S. Securities and Exchange Commission, U.S. financial reports of the Company, and all other documents that the Company is required to deliver to its security holders (including, without limitation, annual reports and proxy statements), or other communications or information related to the RSUs and current or future participation in the Plan. Electronic delivery may include the delivery of a link to the Company intranet or the internet site of a third party involved in administering the Plan, the delivery of the document via e-mail, or such other delivery determined at the Company's discretion. Participant acknowledges that Participant may receive from the Company a paper copy of any documents delivered electronically at no cost if Participant contacts the Company by telephone, through a postal service, or electronic mail to Stock Administration. Participant further acknowledges that Participant will be provided with a paper copy of any documents delivered electronically if electronic delivery fails; similarly, Participant understands that Participant must provide on request to the Company or any designated third party a paper copy of any documents delivered electronically if electronic delivery fails. Also, Participant understands that Participant's consent may be revoked or changed, including any change in the electronic mail address to which documents are delivered (if Participant has provided an electronic mail address), at any time by notifying the Company of such revised or revoked consent by telephone, postal service, or electronic mail to Stock Administration. Finally, Participant understands that Participant is not required to consent to electronic delivery if local laws prohibit such consent.

20. Insider Trading Restrictions/Market Abuse Laws. Participant acknowledges that, depending on Participant's country, Participant may be subject to insider trading restrictions and/or market abuse laws, which may affect Participant's ability to acquire or sell the Shares or rights to Shares under the Plan during such times as Participant is considered to have "inside information" regarding the Company (as defined by the laws in Participant's country). Any restrictions under these laws or regulations are separate from and in addition to any restrictions that may be imposed under any applicable Company insider trading policy. Participant acknowledges that it is Participant's responsibility to comply with any applicable restrictions and understands that Participant should consult his or her personal legal advisor on such matters. In addition, Participant acknowledges that he or she read the Company's Insider Trading Policy, and agrees to comply with such policy, as it may be amended from time to time, whenever Participant acquires or disposes of the Company's securities.

21. Code Section 409A. For purposes of this Agreement, a termination of employment will be determined consistent with the rules relating to a "separation from service" as defined in Section 409A of the Internal Revenue Code and the regulations thereunder ("***Section 409A***"). Notwithstanding anything else provided herein, to the extent any payments provided under this RSU Agreement in connection with Participant's termination of employment constitute deferred compensation subject to Section 409A, and Participant is deemed at the time of such termination of employment to be a "specified employee" under Section 409A, then such payment will not be made or commence until the earlier of (a) the expiration of the six (6) month period measured from Participant's separation from service to the Employer or the Company, or (b) the date of Participant's death following such a separation from service; provided, however, that such deferral will only be effected to the extent required to avoid adverse tax treatment to Participant including, without limitation, the additional tax for which Participant would otherwise be liable under Section 409A(a)(1)(B) in the absence of such a deferral. To the extent any payment under this RSU Agreement may be classified as a "short-term deferral" within the meaning of Section 409A, such payment will be deemed a short-term deferral, even if it may also qualify for an exemption from Section 409A under another provision of Section 409A. Payments pursuant to this section are intended to constitute separate payments for purposes of Section 1.409A-2(b)(2) of the Treasury Regulations.

22. Award Subject to Company Clawback or Recoupment. To the extent permitted by applicable law, the RSUs will be subject to clawback or recoupment pursuant to any compensation clawback or

recoupment policy adopted by the Board or required by law during the term of Participant's employment or other Service that is applicable to Participant. In addition to any other remedies available under such policy and applicable law, the Company may require the cancellation of Participant's RSUs (whether vested or unvested) and the recoupment of any gains realized with respect to Participant's RSUs.

BY ACCEPTING THIS AWARD OF RSUS, PARTICIPANT AGREES TO ALL OF THE TERMS AND CONDITIONS DESCRIBED ABOVE AND IN THE PLAN.

APPENDIX

STOKE THERAPEUTICS, INC.
2019 EQUITY INCENTIVE PLAN
RESTRICTED STOCK UNIT AWARD AGREEMENT

COUNTRY SPECIFIC PROVISIONS FOR EMPLOYEES OUTSIDE THE U.S.

Terms and Conditions

This Appendix includes additional terms and conditions that govern the RSUs granted to Participant under the Plan if Participant resides and/or works in one of the countries below. This Appendix forms part of the Agreement. Any capitalized term used in this Appendix without definition will have the meaning ascribed to it in the Notice, the Agreement, or the Plan, as applicable.

If Participant is a citizen or resident of a country, or is considered resident of a country, other than the one in which Participant is currently working, or Participant transfers employment and/or residency between countries after the Date of Grant, the Company will, in its sole discretion, determine to what extent the additional terms and conditions included herein will apply to Participant under these circumstances.

Notifications

This Appendix also includes information relating to exchange control, securities laws, foreign asset/account reporting, and other issues of which Participant should be aware with respect to Participant's participation in the Plan. The information is based on the securities, exchange control, foreign asset/account reporting, and other laws in effect in the respective countries as of [ ]. Such laws are complex and change frequently. As a result, Participant should not rely on the information herein as the only source of information relating to the consequences of Participant's participation in the Plan because the information may be out of date at the time that Participant vests in the RSUs, sells Shares acquired under the Plan, or takes any other action in connection with the Plan.

In addition, the information is general in nature and may not apply to Participant's particular situation, and the Company is not in a position to assure Participant of any particular result. Accordingly, Participant should seek appropriate professional advice as to how the relevant laws in Participant's country may apply to Participant's situation.

Finally, if Participant is a citizen or resident of a country, or is considered resident of a country, other than the one in which Participant is currently working and/or residing, or Participant transfers employment and/or residency after the Date of Grant, the information contained herein may not apply to Participant in the same manner.

APPENDIX

STOKE THERAPEUTICS, INC.
2019 EQUITY INCENTIVE PLAN
RESTRICTED STOCK UNIT AWARD AGREEMENT

COUNTRY SPECIFIC PROVISIONS FOR EMPLOYEES OUTSIDE THE U.S.

None

STOKE THERAPEUTICS, INC.
2019 EMPLOYEE STOCK PURCHASE PLAN

1. Establishment of Plan. Stoke Therapeutics, Inc., a Delaware corporation (the "***Company***") proposes to grant options to purchase shares of Common Stock to eligible employees of the Company and its Participating Corporations pursuant to this Plan. The Company intends this Plan to qualify as an "employee stock purchase plan" under Code Section 423 (including any amendments to or replacements of such Section), and this Plan will be so construed. Any term not expressly defined in this Plan but defined for purposes of Code Section 423 will have the same definition herein. However, with regard to offers of options for purchase of the Common Stock under the Plan to employees outside the United States working for a Subsidiary or an Affiliate, the Board may offer a subplan or an option that is not intended to meet the Code Section 423 requirements, provided, if necessary under Code Section 423, that the other terms and conditions of the Plan are met. Subject to Section 14, a total of Three Hundred and Fifteen Thousand (315,000) shares of Common Stock is reserved for issuance under this Plan. In addition, on each January 1 for the first ten (10) calendar years after the first Offering Date, the aggregate number of shares of Common Stock reserved for issuance under the Plan will be increased automatically by the number of shares equal to one percent (1%) of the total number of outstanding shares of all classes of common stock on the immediately preceding December 31 (rounded down to the nearest whole share); provided, that the Board or the Committee may in its sole discretion reduce the amount of the increase in any particular year; and, provided further, that the aggregate number of shares issued over the term of this Plan will not exceed Three Million One Hundred and Fifty Thousand (3,150,000) shares of Common Stock. The number of shares reserved for issuance under this Plan and the maximum number of shares that may be issued under this Plan will be subject to adjustments effected in accordance with Section 14 of this Plan. Capitalized terms not defined elsewhere in the text are defined in Section 27.

2. Purpose. The purpose of this Plan is to provide eligible employees of the Company and Participating Corporations with a means of acquiring an equity interest in the Company through payroll deductions, to enhance such employees' sense of participation in the affairs of the Company and Participating Corporations, and to provide an incentive for continued employment.

3. Administration.

(a) The Plan will be administered by the Compensation Committee of the Board or by the Board (either referred to herein as the "***Committee***"). Subject to the provisions of this Plan and the limitations of Section 423 of the Code or any successor provision in the Code, all questions of interpretation or application of this Plan will be determined by the Committee and its decisions will be final and binding upon all Participants. The Committee will have full and exclusive discretionary authority to construe, interpret, and apply the terms of the Plan, to determine eligibility and determine which entities shall be Participating Corporations, and to decide upon any and all claims filed under the Plan. Every finding, decision, and determination made by the Committee will, to the full extent permitted by law, be final and binding upon all parties. The Committee will have the authority to determine the Fair Market Value of the Common Stock (which determination will be final, binding, and conclusive for all purposes) in accordance with Section 8 below and to interpret Section 8 of the Plan in connection with circumstances that impact the Fair Market Value. Members of the Committee will receive no compensation for their services in connection with the administration of this Plan, other than standard fees as established from time to time by the Board for services rendered by Board members serving on the Board or its committees. All expenses incurred in connection with the administration of this Plan will be paid by the Company. For purposes of this Plan, the Committee may designate separate offerings under the Plan (the terms of which need not be identical) in which eligible employees of one or more Participating Corporations will participate, even if the dates of the applicable Offering Periods of each such offering are identical. The Committee may also establish rules to govern transfers of employment among the Company and any Participating Corporation, consistent with the applicable requirements of Code Section 423 and the terms of the Plan.

(b) The Committee may adopt such rules, procedures, and sub-plans as are necessary or appropriate to permit the participation in the Plan by eligible employees who are citizens or residents of a jurisdiction and/or employed outside the United States, the terms of which sub-plans may take precedence over other provisions of this Plan, with the exception of the provisions in Section 1 above setting forth the number of shares of Common Stock reserved for issuance under the Plan; provided, that unless otherwise superseded by the terms of such sub-plan, the provisions of this Plan shall govern the operation of such sub-plan. Further, the Committee is specifically authorized to adopt rules and procedures regarding the application of the definition of Compensation (as defined in Section 9(a) below) to participants on payrolls outside of the United States, handling of payroll deductions and other contributions, taking of payroll deductions and making of other contributions to the Plan, establishment of bank or trust accounts to hold contributions, payment of interest, establishment of the exchange rate applicable to payroll deductions taken and other contributions made in a currency other than U.S. dollars, obligations to pay payroll tax, determination of beneficiary designation requirements, tax withholding procedures, and handling of stock certificates that vary with applicable local requirements.

4. Eligibility. Any employee of the Company or the Participating Corporations is eligible to participate in an Offering Period under this Plan except the following (other than where prohibited by applicable law):

(a) employees who are not employed by the Company or a Participating Corporation prior to the beginning of such Offering Period or prior to such other time period as specified by the Committee;

(b) employees who are customarily employed for twenty (20) or less hours per week (unless determined otherwise by the Committee);

(c) employees who are customarily employed for five (5) months or less in a calendar year(unless determined otherwise by the Committee);

(d) employees who, together with any other person whose stock would be attributed to such employee pursuant to Section 424(d) of the Code, own stock or hold options to purchase stock possessing five percent (5%) or more of the total combined voting power or value of all classes of stock of the Company or any of its Participating Corporations or who, as a result of being granted an option under this Plan with respect to such Offering Period, would own stock or hold options to purchase stock possessing five percent (5%) or more of the total combined voting power or value of all classes of stock of the Company or any of its Participating Corporations;

(e) employees who do not meet any other eligibility requirements that the Committee may choose to impose (within the limits permitted by the Code and other applicable laws); and

(f) individuals who provide services to the Company or any of its Participating Corporations as independent contractors who are reclassified as common law employees for any reason except for federal income and employment tax purposes.

The foregoing notwithstanding, an individual will not be eligible if his or her participation in the Plan is prohibited by the law of any country that has jurisdiction over him or her, if complying with the laws of the applicable country would cause the Plan to violate Section 423 of the Code, or if he or she is subject to a collective bargaining agreement that does not provide for participation in the Plan.

5. Offering Dates. While the Plan is in effect, the Committee will determine the duration and commencement date of each Offering Period and Purchase Period, provided that an Offering Period will in no event be longer than twenty-seven (27) months, except as otherwise provided by an applicable subplan. Offering Periods may be consecutive or overlapping. Each Offering Period may consist of one or more Purchase Periods during which payroll deductions of Participants are accumulated under this Plan. While the Plan is in effect, the Committee will determine the duration and commencement date of each Offering Period and Purchase Period, provided that a Purchase Period will in no event end later than the close of the Offering Period in which it begins. Purchase Periods will be consecutive. The Committee shall have the power to change these terms as provided in Section 25 below.

6. Participation in this Plan.

(a) **Enrollment**. Any employee who is an eligible employee determined in accordance with Section 4 may elect to become a Participant by submitting a subscription agreement, or electronic representation thereof, to the Company and/or via an authorized third party administrator (the "***Third Party Administrator***")'s standard process, prior to the commencement of the Offering Period to which such agreement relates in accordance with such rules as the Committee may determine.

(b) **Continued Enrollment in Offering Periods**. Once an employee becomes a Participant in an Offering Period, then such Participant will automatically participate in each subsequent Offering Period commencing immediately following the last day of such prior Offering Period at the same contribution level unless the Participant withdraws or is deemed to withdraw from this Plan or terminates further participation in the Offering Period as set forth in Section 11 below or otherwise notifies the Company of a change in the Participant's contribution level by filing an additional subscription agreement or electronic representation thereof with the Company and/or the Third Party Administrator, prior to the next Offering Period. A Participant that is automatically enrolled in a subsequent Offering Period pursuant to this section (i) is not required to file any additional subscription agreement in order to continue participation in this Plan, and (ii) will be deemed to have accepted the terms and conditions of the Plan, any sub-plan, and subscription agreement in effect at the time each subsequent Offering Period begins, subject to Participant's right to withdraw from the Plan in accordance with the withdrawal procedures in effect at the time.

7. Grant of Option on Enrollment. Becoming a Participant with respect to an Offering Period will constitute the grant (as of the Offering Date) by the Company to such Participant of an option to purchase on the Purchase Date up to that number of shares of Common Stock determined by a fraction, the numerator of which is the amount of the contribution level for such Participant multiplied by such Participant's Compensation (as defined in Section 9 below) during such Purchase Period and the denominator of which is the lower of (a) eighty-five percent (85%) of the Fair Market Value of a share of the Common Stock on the Offering Date (but in no event less than the par value of a share of the Company's Common Stock), or (b) eighty-five percent (85%) of the Fair Market Value of a share of the Common Stock on the Purchase Date (but in no event less than the par value of a share of the Common Stock); provided, however, , that the number of shares of Common Stock subject to any option granted pursuant to this Plan will not exceed the lesser of (x) the maximum number of shares set by the Committee pursuant to Section 10(b) below with respect to the applicable Purchase Date, or (y) the maximum number of shares which may be purchased pursuant to Section 10(a) below with respect to the applicable Purchase Date.

8. Purchase Price. The Purchase Price in any Offering Period will be eighty-five percent (85%) of the lesser of:

(a) the Fair Market Value on the Offering Date; or

(b) the Fair Market Value on the Purchase Date.

9. Payment of Purchase Price; Payroll Deduction Changes; Share Issuances.

(a) The Purchase Price of the shares is accumulated by regular payroll deductions made during each Offering Period, unless the Committee determines that contributions may be, or are required to be, made in another form (due to local law requirements, in another form with respect to categories of Participants outside the United States). The deductions are made as a percentage of the Participant's compensation in one percent (1%) increments not less than one percent (1%), nor greater than fifteen percent (15%) or such lower limit set by the Committee. "***Compensation***" means base salary, and regular hourly wages (or in foreign jurisdictions, equivalent cash compensation); however, the Committee may at any time prior to the beginning of an Offering Period determine that for that and future Offering Periods, Compensation means base salary or regular hourly wages, bonuses, cash incentive compensation, sales commissions, overtime, shift premiums, plus draws against commissions (or in foreign jurisdictions, equivalent cash compensation). For purposes of determining a Participant's Compensation, any election by such Participant to reduce his or her regular cash remuneration under Sections 125 or 401(k) of the Code (or in foreign jurisdictions, equivalent salary deductions) will be treated as if the Participant did not make such election. Payroll deductions shall commence on the first payday following the beginning of any Offering Period, and shall continue to the end of the applicable Offering Period unless sooner altered or terminated as provided in this Plan. Notwithstanding the foregoing, the terms of any subplan may permit matching shares without the payment of any purchase price.

(b) Subject to Section 25 below and to the rules of the Committee, a Participant may decrease the rate of payroll deductions during an Offering Period by filing with the Company and/or the Third Party Administrator a new authorization for payroll deductions, with the new rate to become effective as soon as reasonably practicable and continuing for the remainder of the Offering Period unless changed as described below. A decrease in the rate of payroll deductions may be made once during an Offering Period or more or less frequently under rules determined by the Committee. An increase in the rate of payroll deductions may not be made during an Offering Period unless otherwise determined by the Committee. A Participant may increase or decrease the rate of payroll deductions for any subsequent Offering Period by filing with the Company and/or the Third Party Administrator a new authorization for payroll deductions prior to the beginning of such Offering Period, or such other time period as may be specified by the Committee.

(c) Subject to Section 25 below and to the rules of the Committee, a Participant may reduce his or her payroll deduction percentage to zero during an Offering Period by filing with the Company a request for cessation of payroll deductions, and after such reduction becomes effective no further payroll deductions will be made for the duration of the Offering Period. Payroll deductions credited to the Participant's account prior to the effective date of the request will be used to purchase shares of Common Stock in accordance with Section (e) below. A reduction of the payroll deduction percentage to zero will be treated as such Participant's withdrawal from such Offering Period, and the Plan, effective as of the day after the next Purchase Date following the filing date of such request with the Company.

(d) All payroll deductions made for a Participant are credited to his or her account under this Plan and are deposited with the general funds of the Company, and the Company will not be obligated to segregate such payroll deductions, except to the extent required to be segregated due to local legal restrictions outside the United States. No interest accrues on the payroll deductions except to the extent required due to local legal requirements outside the United States. All payroll deductions received or held by the Company may be used by the Company for any corporate purpose, except to the extent necessary to comply with local legal requirements outside the United States.

(e) On each Purchase Date, so long as this Plan remains in effect and provided that the Participant has not submitted a signed and completed withdrawal form before that date which notifies the

Company and/or the Third Party Administrator that the Participant wishes to withdraw from that Offering Period under this Plan and have all payroll deductions accumulated in the account maintained on behalf of the Participant as of that date returned to the Participant, the Company will apply the funds then in the Participant's account to the purchase of whole shares of Common Stock reserved under the option granted to such Participant with respect to the Offering Period to the extent that such option is exercisable on the Purchase Date. The Purchase Price will be as specified in Section 8 of this Plan. Any amount remaining in a Participant's account on a Purchase Date which is less than the amount necessary to purchase a full share of Common Stock will be carried forward into the next Purchase Period or Offering Period, as the case may be (except to the extent required due to local legal requirements outside the United States), or as otherwise determined by the Committee. In the event that this Plan has been oversubscribed, all funds not used to purchase shares on the Purchase Date will be returned to the Participant, without interest (except to the extent required due to local legal requirements outside the United States). No Common Stock will be purchased on a Purchase Date on behalf of any employee whose participation in this Plan has terminated prior to such Purchase Date (except to the extent required due to local legal requirements outside the United States).

(f) As promptly as practicable after the Purchase Date, the Company will issue shares for the Participant's benefit representing the shares purchased upon exercise of his or her option.

(g) During a Participant's lifetime, his or her option to purchase shares hereunder is exercisable only by him or her. The Participant will have no interest or voting right in shares covered by his or her option until such option has been exercised.

(h) To the extent required by applicable federal, state, local, or foreign law, a Participant will make arrangements satisfactory to the Company and the Participating Corporation employing the Participant for the satisfaction of any withholding tax obligations that arise in connection with the Plan. The Company or any Participating Corporation, as applicable, may withhold, by any method permissible under applicable law, the amount necessary for the Company or any Participating Corporation, as applicable, to meet applicable withholding obligations, including up to the maximum permissible statutory rates and including any withholding required to make available to the Company or any Participating Corporation, as applicable, any tax deductions or benefits attributable to the sale or early disposition of shares of Common Stock by a Participant. The Company will not be required to issue any shares of Common Stock under the Plan until such obligations are satisfied.

10. Limitations on Shares to be Purchased.

(a) No Participant will be entitled to purchase stock under any Offering Period at a rate which, when aggregated with such Participant's rights to purchase stock under all other employee stock purchase plans of a Participating Company intended to meet the requirements of Section 423 of the Code, that are also outstanding in the same calendar year(s) (whether under other Offering Periods or other employee stock purchase plans of the Company, its Parent, and its Subsidiaries), exceeds $25,000 in Fair Market Value, determined as of the Offering Date (or such other limit as may be imposed by the Code) for each calendar year in which such Offering Period is in effect (hereinafter the "***Maximum Share Amount***"). The Company may automatically suspend the payroll deductions of any Participant as necessary to enforce such limit provided that when the Company automatically resumes such payroll deductions, the Company must apply the rate in effect immediately prior to such suspension.

(b) The Committee may, in its sole discretion, set a lower maximum number of shares which may be purchased by any Participant during any Offering Period than that determined under Section 10(a) above, which will then be the Maximum Share Amount for subsequent Offering Periods; provided, however, in no event will a Participant be permitted to purchase more than Two Thousand Five Hundred (2,500) Shares during any one Purchase Period or such greater or lesser number as the Committee may

determine, irrespective of the Maximum Share Amount set forth in (a) and (b) hereof. If a new Maximum Share Amount is set, then all Participants will be notified of such Maximum Share Amount prior to the commencement of the next Offering Period for which it is to be effective. The Maximum Share Amount will continue to apply with respect to all succeeding Offering Periods unless revised by the Committee as set forth above.

(c) If the number of shares to be purchased on a Purchase Date by all Participants exceeds the number of shares then available for issuance under this Plan, then the Company will make a pro rata allocation of the remaining shares in as uniform a manner as will be reasonably practicable and as the Committee will determine to be equitable. In such event, the Company will give written notice of such reduction of the number of shares to be purchased under a Participant's option to each Participant affected.

(d) Any payroll deductions accumulated in a Participant's account which are not used to purchase stock due to the limitations in this Section 10, and not covered by Section 9(e), will be returned to the Participant as soon as administratively practicable after the end of the applicable Purchase Period, without interest (except to the extent required due to local legal requirements outside the United States).

11. Withdrawal.

(a) Each Participant may withdraw from an Offering Period under this Plan pursuant to a method specified by the Company. Such withdrawal may be elected at any time prior to the end of an Offering Period, or such other time period as specified by the Committee. The Committee may set forth a deadline of when a withdrawal must occur to be effective prior to a given Purchase Date in accordance with policies it may approve from time to time.

(b) Upon withdrawal from this Plan, the accumulated payroll deductions will be returned to the withdrawn Participant, without interest (except to the extent required due to local legal requirements outside the United States), and his or her interest in this Plan will terminate. In the event a Participant voluntarily elects to withdraw from this Plan, he or she may not resume his or her participation in this Plan during the same Offering Period, but he or she may participate in any Offering Period under this Plan which commences on a date subsequent to such withdrawal by filing a new authorization for payroll deductions in the same manner as set forth in Section 6 above for initial participation in this Plan.

(c) To the extent applicable, if the Fair Market Value on the first day of the current Offering Period in which a participant is enrolled is higher than the Fair Market Value on the first day of any subsequent Offering Period, the Company will automatically enroll such participant in the subsequent Offering Period. Any funds accumulated in a participant's account prior to the first day of such subsequent Offering Period will be applied to the purchase of shares on the Purchase Date immediately prior to the first day of such subsequent Offering Period, if any.

12. Termination of Employment. Termination of a Participant's employment for any reason, including (but not limited to) retirement, death, disability, or the failure of a Participant to remain an eligible employee of the Company or of a Participating Corporation, or Participant's employer no longer being a Participating Corporation, immediately terminates his or her participation in this Plan (except to the extent required due to local legal requirements outside the United States). In such event, accumulated payroll deductions credited to the Participant's account will be returned to him or her or, in the case of his or her death, to his or her legal representative, without interest (except to the extent required due to local legal requirements outside the United States). For purposes of this Section 12, an employee will not be deemed to have terminated employment or failed to remain in the continuous employ of the Company or of a Participating Corporation in the case of sick leave, military leave, or any other leave of absence approved by the Company; provided, that such leave is for a period of not more than ninety (90) days or reemployment

upon the expiration of such leave is guaranteed by contract or statute. The Company will have sole discretion to determine whether a Participant has terminated employment and the effective date on which the Participant terminated employment, regardless of any notice period or garden leave required under local law.

13. Return of Payroll Deductions. In the event a Participant's interest in this Plan is terminated by withdrawal, termination of employment, or otherwise, or in the event this Plan is terminated by the Board, the Company will deliver to the Participant all accumulated payroll deductions credited to such Participant's account. No interest will accrue on the payroll deductions of a Participant in this Plan (except to the extent required due to local legal requirements outside the United States).

14. Capital Changes. If the number of outstanding shares is changed by a stock dividend, recapitalization, stock split, reverse stock split, subdivision, combination, reclassification, or similar change in the capital structure of the Company, without consideration, then the Committee will adjust the number and class of Common Stock that may be delivered under the Plan, the Purchase Price, and the number of shares of Common Stock covered by each option under the Plan which has not yet been exercised, and the numerical limits of Sections 1 and 10 will be proportionately adjusted, subject to any required action by the Board or the stockholders of the Company and in compliance with applicable securities laws; provided, that fractions of a Share will not be issued.

15. Nonassignability. Neither payroll deductions credited to a Participant's account nor any rights with regard to the exercise of an option or to receive shares under this Plan may be assigned, transferred, pledged, or otherwise disposed of in any way (other than by will, pursuant to the laws of descent and distribution, or as provided in Section 22 below) by the Participant. Any such attempt at assignment, transfer, pledge, or other disposition will be void and without effect.

16. Use of Participant Funds and Reports. The Company may use all payroll deductions received or held by it under the Plan for any corporate purpose, and the Company will not be required to segregate Participant payroll deductions (except to the extent required due to local legal requirements outside the United States). Until shares are issued, Participants will only have the rights of an unsecured creditor (except to the extent required due to local legal requirements outside the United States). Each Participant will receive, or have access to, promptly after the end of each Purchase Period a report of his or her account setting forth the total payroll deductions accumulated, the number of shares purchased, the Purchase Price thereof, and the remaining cash balance, if any, carried forward or refunded, as determined by the Committee, to the next Purchase Period or Offering Period, as the case may be.

17. Notice of Disposition. If Participant is subject to tax in the United States, Participant will notify the Company in writing if the Participant disposes of any of the shares purchased in any Offering Period pursuant to this Plan if such disposition occurs within two (2) years from the Offering Date or within one (1) year from the Purchase Date on which such shares were purchased (the "***Notice Period***"). The Company may, at any time during the Notice Period, place a legend or legends on any certificate representing shares acquired pursuant to this Plan requesting the Company's transfer agent to notify the Company of any transfer of the shares. The obligation of the Participant to provide such notice will continue notwithstanding the placement of any such legend on the certificates.

18. No Rights to Continued Employment. Neither this Plan nor the grant of any option hereunder will confer any right on any employee to remain in the employ of the Company or any Participating Corporation, or restrict the right of the Company or any Participating Corporation to terminate such employee's employment.

19. Equal Rights And Privileges. All eligible employees granted an option under this Plan that is intended to meet the Code Section 423 requirements will have equal rights and privileges with respect to this Plan or within any separate offering under the Plan so that this Plan qualifies as an "employee stock purchase plan" within the meaning of Section 423 or any successor provision of the Code and the related regulations. Any provision of this Plan which is inconsistent with Section 423 or any successor provision of the Code will, without further act or amendment by the Company or the Committee, be reformed to comply with the requirements of Section 423 (unless such provision applies exclusively to options that granted under the Plan that are not intended to comply with the Code Section 423 requirements). This Section 19 will take precedence over all other provisions in this Plan.

20. Notices. All notices or other communications by a Participant to the Company under or in connection with this Plan will be deemed to have been duly given when received in the form specified by the Company at the location, or by the person, designated by the Company for the receipt thereof.

21. Term; Stockholder Approval. This Plan will become effective on the Effective Date. This Plan will be approved by the stockholders of the Company within twelve (12) months before or after the date this Plan is adopted by the Board. No purchase of shares that are subject to such stockholder approval before becoming available under this Plan will occur prior to stockholder approval of such shares, and the Committee may delay any Purchase Date and postpone the commencement of any Offering Period subsequent to such Purchase Date as deemed necessary or desirable to obtain such approval (provided, that if a Purchase Date would occur more than twenty-four (24) months after commencement of the Offering Period to which it relates, then such Purchase Date will not occur, and instead such Offering Period will terminate without the purchase of such shares and Participants in such Offering Period will be refunded their contributions without interest unless the payment of interest is required under local laws). This Plan will continue until the earlier to occur of (a) termination of this Plan by the Board (which termination may be effected by the Board at any time pursuant to Section 25 below), (b) issuance of all of the shares of Common Stock reserved for issuance under this Plan, or (c) the tenth anniversary of the first Purchase Date under the Plan.

22. Designation of Beneficiary.

(a) If provided in the subscription agreement, a Participant may file a written or electronic designation of a beneficiary who is to receive any shares and cash, if any, from the Participant's account under this Plan in the event of such Participant's death subsequent to the end of a Purchase Period but prior to delivery to him of such shares and cash. In addition, a Participant may file a written or electronic designation of a beneficiary who is to receive any cash from the Participant's account under this Plan in the event of such Participant's death prior to a Purchase Date. Such form will be valid only if it was filed with the Company and/or the Third Party Administrator at the prescribed location before the Participant's death.

(b) Such designation of beneficiary may be changed by the Participant at any time by written notice filed with the Company at the prescribed location before the Participant's death. In the event of the death of a Participant, and in the absence of a beneficiary validly designated under this Plan who is living at the time of such Participant's death, the Company will deliver such cash to the executor or administrator of the estate of the Participant, or if no such executor or administrator has been appointed (to the knowledge of the Company), the Company, in its discretion, may deliver such shares or cash to the spouse or, if no spouse is known to the Company, then to any one or more dependents or relatives of the Participant, or if no spouse, dependent or relative is known to the Company, then to such other person as the Company may designate.

23. Conditions Upon Issuance of Shares; Limitation on Sale of Shares. Shares will not be issued with respect to an option unless the exercise of such option and the issuance and delivery of such

shares pursuant thereto will comply with all applicable provisions of law, domestic or foreign, including, without limitation, the Securities Act, the Exchange Act, the rules and regulations promulgated thereunder, and the requirements of any stock exchange or automated quotation system upon which the shares may then be listed, exchange control restrictions, securities law restrictions, or other applicable laws outside the United States, and will be further subject to the approval of counsel for the Company with respect to such compliance. Shares may be held in trust or subject to further restrictions as permitted by any subplan.

24. Applicable Law. The Plan will be governed by the substantive laws (excluding the conflict of laws rules) of the State of Delaware.

25. Amendment or Termination. The Committee, in its sole discretion, may amend, suspend, or terminate the Plan, or any part thereof, at any time and for any reason. If the Plan is terminated, the Committee, in its discretion, may elect to terminate all outstanding Offering Periods either immediately or upon completion of the purchase of shares of Common Stock on the next Purchase Date (which may be sooner than originally scheduled, if determined by the Committee in its discretion), or may elect to permit Offering Periods to expire in accordance with their terms (and subject to any adjustment pursuant to Section 14). If an Offering Period is terminated prior to its previously-scheduled expiration, all amounts then credited to Participants' accounts for such Offering Period, which have not been used to purchase shares of Common Stock, will be returned to those Participants (without interest thereon, except as otherwise required under local laws) as soon as administratively practicable. Further, the Committee will be entitled to establish rules to change the Purchase Periods and Offering Periods, limit the frequency and/or number of changes in the amount contributed during a Purchase Period or an Offering Period, establish the exchange ratio applicable to amounts contributed in a currency other than U.S. dollars, permit payroll withholding in excess of the amount designated by a Participant in order to adjust for delays or mistakes in the administration of the Plan, establish reasonable waiting and adjustment periods and/or accounting and crediting procedures to ensure that amounts applied toward the purchase of Common Stock for each Participant properly correspond with amounts contributed from the Participant's Compensation, and establish such other limitations or procedures as the Committee determines in its sole discretion advisable which are consistent with the Plan. Such actions will not require stockholder approval or the consent of any Participants. However, no amendment will be made without approval of the stockholders of the Company (obtained in accordance with Section 21 above) within twelve (12) months of the adoption of such amendment (or earlier if required by Section 21) if such amendment would (a) increase the number of shares that may be issued under this Plan or (b) change the designation of the employees (or class of employees) eligible for participation in this Plan. In addition, in the event the Committee determines that the ongoing operation of the Plan may result in unfavorable financial accounting consequences, the Committee may, in its discretion and, to the extent necessary or desirable, modify, amend, or terminate the Plan to reduce or eliminate such accounting consequences including, but not limited to: (a) amending the definition of compensation, including with respect to an Offering Period underway at the time; (b) altering the Purchase Price for any Offering Period including an Offering Period underway at the time of the change in Purchase Price; (c) shortening any Offering Period by setting a Purchase Date, including an Offering Period underway at the time of the Committee action; (d) reducing the maximum percentage of compensation a participant may elect to set aside as payroll deductions; and (e) reducing the maximum number of shares of Common Stock a Participant may purchase during any Offering Period. Such modifications or amendments will not require approval of the stockholders of the Company or the consent of any Participants.

26. Corporate Transactions. In the event of a Corporate Transaction (as defined below), each outstanding right to purchase Common Stock will be assumed or an equivalent option substituted by the successor corporation or a parent or a subsidiary of the successor corporation. In the event that the successor corporation refuses to assume or substitute for the purchase right, the Offering Period with respect to which such purchase right relates will be shortened by setting a new Purchase Date (the "***New Purchase Date***") and will end on the New Purchase Date. The New Purchase Date will occur on or prior to the consummation of the Corporate Transaction, and the Plan will terminate on the consummation of the Corporate Transaction.

27. Definitions.

(a) "***Affiliate***" means any entity, other than a Subsidiary or Parent, (i) that, directly or indirectly, is controlled by, controls or is under common control with, the Company and (ii) in which the Company has a significant equity interest, in either case as determined by the Committee, whether now or hereafter existing.

(b) "***Board***" means the Board of Directors of the Company.

(c) "***Code***" means the U.S. Internal Revenue Code of 1986, as amended.

(d) "***Common Stock***" means the common stock of the Company.

(e) "***Corporate Transaction***" means the occurrence of any of the following events: (i) any "person" (as such term is used in Sections 13(d) and 14(d) of the Exchange Act) becomes the "beneficial owner" (as defined in Rule 13d-3 of the Exchange Act), directly or indirectly, of securities of the Company representing fifty percent (50%) or more of the total voting power represented by the Company's then outstanding voting securities; (ii) the consummation of the sale or disposition by the Company of all or substantially all of the Company's assets; or (iii) the consummation of a merger or consolidation of the Company with any other corporation, other than a merger or consolidation which would result in the voting securities of the Company outstanding immediately prior thereto continuing to represent (either by remaining outstanding or by being converted into voting securities of the surviving entity or its parent) at least fifty percent (50%) of the total voting power represented by the voting securities of the Company or such surviving entity or its parent outstanding immediately after such merger or consolidation.

(f) "***Effective Date***" means the date on which the Registration Statement covering the initial public offering of the shares of Common Stock is declared effective by the U.S. Securities and Exchange Commission.

(g) "***Exchange Act***" means the U.S. Securities Exchange Act of 1934, as amended.

(h) "***Fair Market Value***" means, as of any date, the value of a share of Common Stock determined as follows:

(i) if such Common Stock is publicly traded and is then listed on a national securities exchange, its closing price on the date of determination on the principal national securities exchange on which the Common Stock is listed or admitted to trading as reported in *The Wall Street* Journal or such other source as the Committee deems reliable;

(ii) if such Common Stock is publicly traded but is neither listed nor admitted to trading on a national securities exchange, the average of the closing bid and asked prices on the date of determination as reported in *The Wall Street Journal* or such other source as the Committee deems reliable;

(iii) if such Common Stock is publicly traded but is neither quoted on the Nasdaq Market nor listed or admitted to trading on a national securities exchange, the average of the closing bid and asked prices on the date of determination as reported in *The Wall Street Journal* or such other source as the Committee deems reliable; and

(iv) if none of the foregoing is applicable, by the Committee in good faith.

(i) "***Offering Date***" means the first Trading Day of each Offering Period.

(j) "***Offering Period***" means a period with respect to which the right to purchase Common Stock may be granted under the Plan, as determined by the Committee pursuant to Section 5.

(k) "***Parent***" will have the same meaning as "parent corporation" in Sections 424(e) and 424(f) of the Code.

(l) "***Participant***" means an eligible employee who meets the eligibility requirements set forth in Section 4 and who elects to participate in this Plan subject and pursuant to Section 6.

(m) "***Participating Corporation***" means any Parent, Subsidiary or Affiliate that the Board designates from time to time as a corporation that will participate in this Plan.

(n) "***Plan***" means this Stoke Therapeutics, Inc. 2019 Employee Stock Purchase Plan.

(o) "***Purchase Date***" means the last Trading Day of each Purchase Period.

(p) "***Purchase Period***" means a period during which contributions may be made toward the purchase of Common Stock under the Plan, as determined by the Committee pursuant to Section 5(b).

(q) "***Purchase Price***" means the price at which Participants may purchase a share of Common Stock under the Plan, as determined pursuant to Section 8.

(r) "***Securities Act***" means the U.S. Securities Act of 1933, as amended.

(s) "***Subsidiary***" will have the same meaning as "subsidiary corporation" in Sections 424(e) and 424(f) of the Code.

(t) "***Trading Day***" means a day on which the national stock exchange upon which the Common Stock is listed is open for trading.

STOKE THERAPEUTICS, INC. (THE "*COMPANY*")
2019 EMPLOYEE STOCK PURCHASE PLAN

ENROLLMENT/CHANGE FORM

SECTION 1:	**CHECK DESIRED ACTION:**	**AND COMPLETE SECTIONS:**
ACTIONS	☐ Enroll in the ESPP	2 + 3 + 4 + 16
	☐ Elect / Change Contribution Percentage	2 + 4 + 16
	☐ Withdraw from Plan	2 + 5 + 16
SECTION 2:	Name: _____	Employee ID:
PERSONAL DATA	Home Address: _____	_____
	_____	
	Work Email: _____	

SECTION 3:

ENROLL

☐ I hereby elect to participate in the 2019 Employee Stock Purchase Plan, together with any sub-plan thereto for my country of residence (if any) (the "***Sub-Plan***") (together, the "***ESPP***"), effective at the beginning of the next Offering Period (as defined in the ESPP). I elect to purchase shares of Common Stock of the Company pursuant to the ESPP, this Enrollment/Change Form, and any appendix to this Enrollment/Change Form for my country (if any) (the "***Appendix***"). I understand that the shares purchased on my behalf will be issued in street name and deposited directly into my brokerage account at the Company's captive broker (the "***ESPP Broker***"). I hereby agree to take all steps, and sign all forms, required to establish an account with the ESPP Broker for this purpose.

My participation will continue as long as I remain eligible, unless I withdraw from the ESPP by filing a new Enrollment/Change Form with the Company and/or the Third Party Administrator (as defined in the ESPP). I understand that, if I am subject to tax in the U.S., I must notify the Company of any disposition of shares purchased under the ESPP.

SECTION 4:

ELECT/CHANGE CONTRIBUTION PERCENTAGE

I hereby authorize the Company or the Parent, Subsidiary, or Affiliate employing me (the "***Employer***") to withhold from each of my paychecks such amount as is necessary to equal at the end of the applicable Offering Period the percentage of my Compensation (as defined in the ESPP) paid to me during such Offering Period as indicated below, so long as I continue to participate in the ESPP. **The percentage must be a whole number (from 1%, up to a maximum of 15%, with respect to enrollment or an increase in contribution percentage; and from 0%, up to a maximum of 14%, for a decrease in contribution percentage).**

Designated contribution percentage: %

If this is a change to my current enrollment, this represents an ☐ increase ☐ decrease to my contribution percentage.

Note: You may not increase your contributions at any time within an on-going Offering Period. An increase in your contribution percentage can only take effect with the next Offering Period. You may decrease your contribution percentage to a percentage other than 0% only once within an ongoing Offering Period to be effective during that Offering Period. If you decrease your percentage to 0%, any previously accumulated contributions will be used to purchase shares on the next Purchase Date pursuant to Section 9 of the ESPP. A change will become effective as soon as reasonably practicable after the form is received by the Company.

SECTION 5:

WITHDRAW FROM PLAN

☐ I hereby elect to withdraw from, and discontinue my participation in, the ESPP, effective as soon as reasonably practicable after this form is received by the Company. Accumulated contributions will be returned to me without interest (except to the extent required due to local legal requirements outside the United States), pursuant to Section 11 of the ESPP.

SECTION 6:

NATURE OF GRANT

By enrolling in the ESPP, I understand, acknowledge, and agree that (a) the ESPP is established voluntarily by the Company, it is discretionary in nature and it may be amended, terminated or modified at any time, to the extent permitted by the ESPP; (b) the grant of the right to purchase shares of Common Stock under the ESPP is voluntary and does not create any contractual or other right to receive future rights to purchase shares of Common Stock, or benefits in lieu of rights to purchase shares, even if rights to purchase shares have been granted in the past; (c) all decisions with respect to future grants of rights to purchase shares of Common Stock under the ESPP, if any, will be at the sole discretion of the Company; (d) the grant of rights to purchase shares of Common Stock under the ESPP and my participation in the ESPP shall not create a right to employment or be interpreted as forming an employment or service agreement with the Company; (e) the grant of rights to purchase shares of Common Stock under the ESPP and my participation in the ESPP shall not interfere with the ability of the Employer to terminate my employment relationship at any time with or without cause; (f) I am voluntarily participating in the ESPP; (g) the rights to purchase shares of Common Stock and the shares purchased under the ESPP, and the income and value of same, are not intended to replace any pension rights or compensation; (h) the rights to purchase shares of Common Stock and the shares purchased under the ESPP, and the income and value of same, are not part of normal or expected compensation for purposes of, including, but not limited to, calculating any severance, resignation, termination, redundancy, dismissal, end of service payments, bonuses, long-service awards, pension or retirement benefits or similar payments; (i) unless otherwise agreed with the Company, the rights to purchase shares of Common Stock and the shares purchased under the ESPP, and the income and value of same, are not granted as consideration for, or in connection with, any service I may provide as a director of the Subsidiary or Affiliate; (j) the future value of the underlying shares purchased or to be purchased under the ESPP is unknown, indeterminable, and cannot be predicted with certainty, and the value of the shares of Common Stock purchased under the ESPP may increase or decrease in the future, even below the Purchase Price; (k) no claim or entitlement to compensation or damages shall arise from termination of the right to purchase shares of Common Stock under the ESPP resulting from termination of my employment (for any reason whatsoever and whether or not later found to be invalid or in breach of employment laws in the jurisdiction where I am employed or the terms of my employment agreement, if any) and in consideration of the grant of rights to purchase shares of Common Stock under the ESPP, I irrevocably agree never to institute any claim against the Company, the Parent, the Employer or any other Subsidiary or Affiliate, I hereby waive my ability, if any, to bring any such claim, and I release the Company, the Parent, the Employer or any other Subsidiary or Affiliate from any such claim; if, notwithstanding the foregoing, any such claim is allowed by a court of competent jurisdiction, then, by enrolling in the ESPP, I shall be deemed irrevocably to have agreed not to pursue such claim and agree to execute any and all documents necessary to request dismissal or withdrawal of such claims; (l) in the event of termination of my employment (for any reason whatsoever, whether or not later found to be invalid or in breach of employment laws in the jurisdiction where I am employed or the terms of my employment agreement, if any), my right to participate in the ESPP and my right to purchase shares of Common Stock, if any, will terminate effective as of the date I cease to actively provide services and will not be extended by any notice period (*e.g.*, employment would not include any contractual notice or any period of "garden leave" or similar period mandated under employment laws in the jurisdiction where I am employed or the terms of my employment agreement, if any); the Committee shall have exclusive discretion to determine when I am no longer actively employed for purposes of my participation in the ESPP (including whether I may still be considered to be providing services while on a leave of absence); (m) unless otherwise provided in the ESPP or by the Company in its discretion, the right to purchase shares of Common Stock and the benefits evidenced by this Enrollment/Change Form do not create any entitlement to have the ESPP or any such benefits granted thereunder transferred to, or assumed by, another company nor to be exchanged, cashed out or substituted for, in connection with any Corporate Transaction affecting the Common Stock; and (n) if I am providing services outside the United States: (1) the rights to purchase shares of Common Stock and the shares purchased under the ESPP, and the income and value of same, are not part of normal or expected compensation or salary for any purpose, and (2) neither the Company, the Parent, the Employer nor any other Subsidiary or Affiliate shall be liable for any foreign exchange rate fluctuation between my local currency and the United States Dollar that may affect the value of the rights to purchase shares of Common Stock, the shares purchased under the ESPP or any amounts due to me pursuant to the sale of any shares of Common Stock acquired under the ESPP.

SECTION 7:

DATA PRIVACY

I understand that the Company and its Parent, Subsidiaries, or Affiliates need to collect and use certain personal information about me (known as "personal data") in order to administer and manage my participation in the ESPP. For the purposes of data protection law, my employer and the Company will be the relevant data controllers. This personal data may include, but may not be limited to, my name, home address and telephone number, email address, date of birth, social insurance number or other identification number, salary, nationality, job title, any shares or directorships held in the Company, details of my participation in the ESPP or any other entitlement to shares awarded, canceled, vested, unvested or outstanding in my favor, for the purpose of implementing, administering and managing the ESPP ("***Data***"). The Data will be processed for the purposes of managing my participation in the ESPP, for example, to maintain a record of outstanding awards, contribution rates, to provide shares on Purchase Dates, to enable relevant information to be supplied to taxation authorities, to enable relevant tax deductions to be made in relation to share awards, and to contact me in relation to events which affect my participation in the ESPP ("***Share Plan Purposes***"). The Data processed for Share Plan Purposes will be gathered: (a) from me directly, and/or (b) by the Company (or its Parent, Subsidiary, or Affiliate that employs me) from my human resources or personnel files. I understand that the Data may also be held by the Company and its Parent, Subsidiaries, or Affiliates for other purposes associated with my employment (which are or will be described in separate privacy notices or policies). Processing the Data for Share Plan Purposes is, in most respects, necessary in order to perform this Enrollment/Change Form. In certain cases, processing will instead be based on the legitimate interests of one or more members of the Company and its Parent, Subsidiaries, or Affiliates in processing the Data for the Share Plan Purposes, in order to deliver a benefit to incentivize and reward its employees. Finally, the Company and its Parent, Subsidiaries, or Affiliates may be required to carry out certain processing activities in order to comply with legal obligations to which it is subject. I understand that Data may be transferred between members of the Company and its Parent, Subsidiaries, or Affiliates and to third parties assisting in the implementation, administration and management of the ESPP (such as brokers and share plan administrators). These recipients may be located in my country or elsewhere, and the recipient's country may have different or less stringent data privacy laws and protections than my country. Where required by law (for example, when Data is transferred outside of the European Economic Area), the Company and its Parent, Subsidiaries, or Affiliates will put in place arrangements (for example, data transfer agreements) to ensure the adequate protection of the Data; non-proprietary or confidential details of such safeguards will be made available to me upon my written request to the Company. I understand that Data will be held by the Company or its Parent, Subsidiaries, or Affiliates for the period specified in its records retention policy. I understand that I have certain rights in respect of the Data, including to access the data, to request erasure of the Data (where no legal basis to continue processing it exists) or to limit or object to processing, to request corrections to inaccurate Data, and to data portability. To exercise any of these rights, or where I have any queries about the processing of their Data, I should contact: [ ]. The data protection contact for the Company and its Parent, Subsidiaries, and Affiliates can be contacted directly: [ ]. I further understand that I have the right to lodge a complaint with a supervisory authority in connection with the violation of the foregoing rights by the Company and its Parent, Subsidiaries, and Affiliates.

SECTION 8:

RESPONSIBILITY FOR TAXES

I acknowledge that, regardless of any action taken by the Company or the Employer, the ultimate liability for all income tax, social insurance, payroll tax, fringe benefits tax, payment on account, or other tax-related items related to my participation in the ESPP and legally applicable to me ("***Tax-Related Items***") is and remains my responsibility and may exceed the amount actually withheld by the Company or the Employer. I further acknowledge that the Company and/or the Employer (a) make no representations or undertakings regarding the treatment of any Tax-Related Items in connection with any aspect of the ESPP, including, but not limited to, my enrollment in the ESPP, the grant of rights to purchase shares of Common Stock, the purchase of shares of Common Stock, the issuance of Common Stock purchased, the sale of shares of Common Stock purchased under the ESPP or the receipt of any dividends; and (b) do not commit to and are under

no obligation to structure the terms of the ESPP to reduce or eliminate my liability for Tax-Related Items or achieve any particular tax result. Further, if I am subject to Tax-Related Items in more than one jurisdiction, I acknowledge that the Company and/or the Employer (or former employer, as applicable) may be required to withhold or account for Tax-Related Items in more than one jurisdiction.

Prior to any relevant taxable or tax withholding event, as applicable, I agree to make adequate arrangements satisfactory to the Company and/or the Employer to satisfy all Tax-Related Items. In this regard, I authorize the Company and/or the Employer to satisfy their withholding obligations with regard to all Tax-Related Items by one or a combination of the following: (a) withholding from my wages or other cash compensation payable to me by the Company and/or the Employer, (b) withholding from proceeds of the sale of shares of Common Stock purchased under the ESPP, either through a voluntary sale or through a mandatory sale arranged by the Company (on my behalf pursuant to this authorization without further consent), and (c) withholding in shares to be issued upon purchase under the ESPP.

Depending on the withholding method, the Company may withhold or account for Tax-Related Items by considering applicable statutory withholding amounts or other applicable withholding rates, including up to the maximum permissible statutory rates, in which case I will receive a refund of any over-withheld amount in cash and will have no entitlement to the Common Stock equivalent. If the obligation for Tax-Related Items is satisfied by withholding in shares of Common Stock, for tax purposes, I am deemed to have been issued the full number of shares of Common Stock, notwithstanding that a number of the shares of Common Stock are held back solely for the purpose of paying the Tax-Related Items.

Finally, I agree to pay to the Company or the Employer any amount of Tax-Related Items that the Company or the Employer may be required to withhold or account for as a result of my participation in the ESPP that cannot be satisfied by the means previously described. The Company may refuse to purchase or deliver the shares or the proceeds from the sale of shares of Common Stock, if I fail to comply with my obligations in connection with the Tax-Related Items.

SECTION 9:

GOVERNING LAW & LANGUAGE

The rights to purchase shares and the provisions of this Enrollment/Change Form are governed by, and subject to, the laws of the State of Delaware, without regard to any conflict of law provisions.

If I have received this or any other document related to the ESPP translated into a language other than English and if the meaning of the translated version is different than the English version, the English version will control.

SECTION 10:

APPENDIX & IMPOSITION OF OTHER REQUIREMENTS

Notwithstanding any provision herein, my participation in the ESPP shall be subject to any special terms and conditions as set forth in the Appendix for my country, if any. Moreover, if I relocate to one of the countries included in the Appendix, the special terms and conditions for such country will apply to me, to the extent the Company determines that the application of such terms and conditions is necessary or advisable for legal or administrative reasons. The Appendix constitutes part of this Enrollment/Change Form.

The Company reserves the right to impose other requirements on my participation in the ESPP or on any shares of Common Stock purchased under the ESPP, to the extent the Company determines it is necessary or advisable for legal or administrative reasons, and to require me to sign any additional agreements or undertakings that may be necessary to accomplish the foregoing.

SECTION 11:

ELECTRONIC DELIVERY AND ACCEPTANCE

The Company may, in its sole discretion, decide to deliver any documents related to current or future participation in the ESPP by electronic means. I hereby consent to receive such documents by electronic delivery and agree to participate in the ESPP through an on-line or electronic system established and maintained by the Company or a third party designated by the Company.

SECTION 12:

SEVERABILITY & WAIVER

The provisions of this Enrollment/Change Form are severable and if any one or more provisions are determined to be illegal or otherwise unenforceable, in whole or in part, the remaining provisions shall nevertheless be binding and enforceable. I acknowledge that a waiver by the Company of breach of any provision of this Enrollment/Change Form shall not operate or be construed as a waiver of any other provision herein, or of any subsequent breach by me or any other Participant.

SECTION 13: **INSIDER TRADING RESTRICTIONS/MARKET ABUSE LAWS**	I acknowledge that I may be subject to insider trading restrictions and/or market abuse laws, which may affect my ability to acquire or sell shares of Common Stock or my rights to purchase shares under the ESPP during such times as I am considered to have "inside information" regarding the Company (as defined by or determined under applicable law). Any restrictions under these laws or regulations are separate from and in addition to any restrictions that may be imposed under any applicable Company insider trading policy. I acknowledge that it is my responsibility to comply with any applicable restrictions, and that I am advised to speak to my personal advisor on this matter.
SECTION 14: **NO ADVICE REGARDING GRANT**	The Company is not providing any tax, legal, or financial advice, nor is the Company making any recommendations regarding my participation in the ESPP, or my purchase or sale of the shares of Common Stock. I am hereby advised to consult with my own personal tax, legal, and financial advisors regarding my participation in the ESPP before taking any action related to the ESPP.
SECTION 15: **COMPLIANCE WITH LAW**	Unless there is an available exemption from any registration, qualification, or other legal requirement applicable to the shares of Common Stock, the Company shall not be required to deliver any shares under the ESPP prior to the completion of any registration or qualification of the shares under any local, state, federal, or foreign securities or exchange control law or under rulings or regulations of the U.S. Securities and Exchange Commission ("***SEC***") or of any other governmental regulatory body, or prior to obtaining any approval or other clearance from any local, state, federal, or foreign governmental agency, which registration, qualification, or approval the Company shall, in its absolute discretion, deem necessary or advisable. I understand that the Company is under no obligation to register or qualify the shares with the SEC or any state or foreign securities commission or to seek approval or clearance from any governmental authority for the issuance or sale of the shares. Further, I agree that the Company shall have unilateral authority to amend the ESPP and the Enrollment/Change Form without my consent to the extent necessary to comply with securities or other laws applicable to issuance of shares.
SECTION 16: **ACKNOWLEDGMENT AND SIGNATURE**	I acknowledge that I have received a copy of the ESPP Prospectus (which summarizes the major features of the ESPP). I have read the Prospectus and my signature below indicates that I hereby agree to be bound by the terms of the ESPP. Signature: _____ Date: _____

APPENDIX

STOKE THERAPEUTICS, INC.
2019 EMPLOYEE STOCK PURCHASE PLAN
ENROLLMENT/CHANGE FORM

COUNTRY SPECIFIC PROVISIONS FOR EMPLOYEES OUTSIDE THE U.S.

None

EXHIBIT 23.1

Consent of Independent Registered Public Accounting Firm

The Board of Directors
Stoke Therapeutics, Inc:

We consent to the use of our report dated March 26, 2019, except as to notes 1 and 14, as to which the date is June 7, 2019, included herein and to the reference to our firm under the heading “Experts” in the prospectus.

/s/ KPMG LLP

Boston, MA
June 7, 2019