UNITED STATES
SECURITIES AND EXCHANGE COMMISSION
WASHINGTON, DC 20549

FORM 10-Q

(Mark One)

☒ **QUARTERLY REPORT PURSUANT TO SECTION 13 OR 15(d) OF THE SECURITIES EXCHANGE ACT OF 1934**

For the quarterly period ended March 31, 2021

OR

☐ **TRANSITION REPORT PURSUANT TO SECTION 13 OR 15(d) OF THE SECURITIES EXCHANGE ACT OF 1934**

For the transition period from _____ to _____

Commission File Number: 001-38938

Stoke Therapeutics, Inc.

(Exact Name of Registrant as Specified in its Charter)

Delaware		**47-1144582**
(State or other jurisdiction of incorporation or organization)		**(I.R.S. Employer Identification No.)**
45 Wiggins Ave **Bedford, Massachusetts**		**01730**
(Address of principal executive offices)		**(Zip Code)**

(781) 430-8200

(Registrant's telephone number, including area code)

Not applicable

(Former name, former address and former fiscal year, if changed since last report)

Securities registered pursuant to Section 12(b) of the Act:

Title of each class	Trading Symbol(s)	Name of each exchange on which registered
Common Stock, $0.0001 par value per share	STOK	Nasdaq Global Select Market

Indicate by check mark whether the registrant (1) has filed all reports required to be filed by Section 13 or 15(d) of the Securities Exchange Act of 1934 during the preceding 12 months (or for such shorter period that the registrant was required to file such reports), and (2) has been subject to such filing requirements for the past 90 days. Yes ☒ No ☐

Indicate by check mark whether the registrant has submitted electronically every Interactive Data File required to be submitted pursuant to Rule 405 of Regulation S-T (§232.405 of this chapter) during the preceding 12 months (or for such shorter period that the registrant was required to submit such files). Yes ☒ No ☐

Indicate by check mark whether the registrant is a large accelerated filer, an accelerated filer, a non-accelerated filer, smaller reporting company, or an 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 pursuant to Section 13(a) of the Exchange Act. ☐

Indicate by check mark whether the registrant is a shell company (as defined in Rule 12b-2 of the Exchange Act). Yes ☐ No ☒

As of May 3, 2021 the registrant had 36,707,526 shares of common stock, $0.0001 par value per share, outstanding.

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FORWARD-LOOKING STATEMENTS

This Quarterly Report on Form 10-Q contains forward-looking statements within the safe harbor provisions for forward-looking statements contained in Section 27A of the Securities Act of 1933, as amended (the "Securities Act") and Section 21E of the Securities Exchange Act of 1934, as amended (the "Exchange Act"). All statements other than statements of present and historical facts contained in this Quarterly Report on Form 10-Q, including statements regarding our future results of operations and financial position, business strategy, prospective products, planned preclinical studies and clinical or field trials, regulatory approvals, research and development costs, and timing and likelihood of success, as well as plans and objectives of management for future operations, may be forward-looking statements. In some cases, you can identify forward-looking statements by terms such as "may," "will," "should," "expect," "plan," "anticipate," "could," "intend," "target," "project," "contemplate," "believe," "estimate," "predict," "potential" or "continue" or the negative of these terms or other similar expressions, although not all forward-looking statements contain these words.

Forward-looking statements are based on our management's beliefs and assumptions and on information currently available to us. Such statements are subject to a number of known and unknown risks, uncertainties and assumptions, and actual results may differ materially from those expressed or implied in the forward-looking statements due to various factors, including, but not limited to, those identified in Part I. Item 2. "Management's Discussion and Analysis of Financial Condition and Results of Operations" and Part II. Item 1A "Risk Factors." These risks and uncertainties include, but are not limited to:

- the direct and indirect impact of COVID-19 on our business, financial condition and operations, including on our expenses, supply chain, strategic partners, research and development costs, clinical trials and employees;
- our ability to become profitable;
- our ability to procure sufficient funding;
- our limited operating history;
- our ability to develop, obtain regulatory approval for and commercialize STK-001 and our future product candidates, including any impact from COVID-19;
- 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 precisely upregulate protein expression in OPA1 protein-deficient cells and to treat the underlying cause of ADOA;
- 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 financial performance; and
- developments or projections relating to our competitors or our industry.

You should read this Quarterly Report on Form 10-Q and the documents that we reference herein completely and with the understanding that our actual future results may be materially different from what we expect. We qualify all of our forward-looking statements by these cautionary statements. Except as required by applicable law, we do not plan to publicly update or revise any forward-looking statements contained herein, whether as a result of any new information, future events, changed circumstances or otherwise.

PART I—FINANCIAL INFORMATION

Item 1. Financial Statements.

Stoke Therapeutics, Inc.
Condensed consolidated balance sheets
(in thousands, except share and per share amounts)
(unaudited)

	March 31, 2021	December 31, 2020
Assets		
Current assets:		
Cash and cash equivalents	$ 267,514	$ 287,308
Prepaid expenses and other current assets	7,511	6,435
Restricted cash - short-term	147	—
Deferred financing costs	117	181
Interest receivable	6	6
Total current assets	$ 275,295	$ 293,930
Restricted cash	58	205
Operating lease right-of-use assets	844	1,115
Property and equipment, net	2,711	2,675
Total assets	$ 278,908	$ 297,925
Liabilities and stockholders' equity		
Current liabilities:		
Accounts payable	$ 1,496	$ 1,495
Accrued and other current liabilities	4,614	9,930
Total current liabilities	$ 6,110	$ 11,425
Long term liabilities	333	422
Total liabilities	$ 6,443	$ 11,847
Commitments and contingencies (Note 5)		
Stockholders' equity		
Common stock, par value of $0.0001 per share; 300,000,000 shares authorized, 36,697,808 and 36,577,149 shares issued and outstanding as of March 31, 2021 and December 31, 2020, respectively	4	4
Additional paid-in capital	399,532	396,352
Accumulated deficit	(127,071)	(110,278)
Total stockholders' equity	$ 272,465	$ 286,078
Total liabilities and stockholders' equity	$ 278,908	$ 297,925

The accompanying notes are an integral part of these unaudited condensed consolidated financial statements.

Stoke Therapeutics, Inc.
Condensed consolidated statements of operations and comprehensive loss
(in thousands, except share and per share amounts)
(unaudited)

	Three Months Ended March 31,	
	2021	2020
Revenue	$ —	$ —
Operating expenses:		
Research and development	9,913	7,215
General and administrative	6,914	4,520
Total operating expenses	16,827	11,735
Loss from operations	(16,827)	(11,735)
Other income:		
Interest income	18	674
Other income (expense), net	16	22
Total other income	34	696
Net loss and comprehensive loss	$ (16,793)	$ (11,039)
Net loss per share, basic and diluted	$ (0.46)	$ (0.34)
Weighted-average common shares outstanding, basic and diluted	36,643,205	32,897,395

The accompanying notes are an integral part of these unaudited condensed consolidated financial statements.

Stoke Therapeutics, Inc.
Condensed consolidated statements of stockholders' equity
(in thousands, except share and per share amounts)
(unaudited)

	Common Stock		Additional paid-in capital	Accumulated deficit	Stockholders' equity
	Shares	Amount	Amount	Amount	Amount
Balance as of December 31, 2019	**32,861,842**	**$ 3**	**$ 282,460**	**$ (58,035)**	**$ 224,428**
Net Loss	—	-	-	(11,039)	(11,039)
Stock-based compensation	—	-	754	-	754
Issuance of common stock upon exercise of stock options	105,518	-	199	-	199
Balance as of March 31, 2020	**32,967,360**	**$ 3**	**$ 283,413**	**$ (69,074)**	**$ 214,342**
Balance as of December 31, 2020	**36,577,149**	**$ 4**	**$ 396,352**	**$ (110,278)**	**$ 286,078**
Net loss	-	-	-	(16,793)	(16,793)
Stock-based compensation	-	-	2,698	-	2,698
Issuance of common stock upon exercise of stock options	111,858	-	371	-	371
Issuance of common stock upon follow-on offering, net of underwriting discounts and offering costs	-	-	(64)	-	(64)
Issuance of common stock related to employee stock purchase plan	8,801	-	175	-	175
Balance as of March 31, 2021	**36,697,808**	**$ 4**	**$ 399,532**	**$ (127,071)**	**$ 272,465**

The accompanying notes are an integral part of these unaudited condensed consolidated financial statements.

Stoke Therapeutics, Inc.
Condensed consolidated statements of cash flows
(in thousands)
(unaudited)

	Three Months Ended March 31,	
	2021	2020
Cash flows from operating activities:		
Net loss	$ (16,793)	$ (11,039)
Adjustments to reconcile net loss to net cash used in operating activities:		
Depreciation	236	193
Stock-based compensation	2,698	754
Loss on disposal of property and equipment	29	3
Reduction in the carrying amount of right of use assets	271	253
Changes in assets and liabilities:		
Prepaid expenses and other current assets	(1,078)	(924)
Accounts payable and accrued liabilities	(5,499)	(11)
Net cash used in operating activities	$ (20,136)	$ (10,771)
Cash flows from investing activities:		
Purchases of property and equipment	(204)	(610)
Net cash used in investing activities	$ (204)	$ (610)
Cash flows from financing activities:		
Proceeds from issuance of common stock upon exercise of stock options	371	199
Proceeds from Employee Stock Purchase Plan	175	—
Payments on capital lease	—	(1)
Net cash provided by financing activities	$ 546	$ 198
Net increase (decrease) in cash, cash equivalents and restricted cash	$ (19,794)	$ (11,183)
Cash, cash equivalents and restricted cash—beginning of period	$ 287,513	$ 222,676
Cash, cash equivalents and restricted cash—end of period	$ 267,719	$ 211,493
Supplemental Disclosure of Non-Cash Investing and Financing Activities:		
Right-of-use assets recognized in exchange for operating leases upon adoption of Topic 842	$ -	$ 2,153
Property and equipment included in accrued expense and accounts payable	$ 97	$ 35

The accompanying notes are an integral part of these unaudited condensed consolidated financial statements.

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 a biotechnology company dedicated to addressing the underlying cause of severe diseases by up-regulating protein expression with RNA-based medicines.

Shelf Registration

In July 2020, the Company filed a universal shelf registration statement on Form S-3 (the "Registration Statement") with the SEC. The Registration Statement was declared effective by the SEC in July 2020, and covers the offering, issuance, and sale by the Company of up to a maximum aggregate offering price of $400,000,000 of our common stock, preferred stock, debt securities, warrants to purchase the Company's common stock, preferred stock or debt securities, subscription rights to purchase the Company's common stock, preferred stock or debt securities and/or units consisting of some or all of these securities. In July 2020, the Company entered into an "at-the-market" program and sales agreement with Cantor Fitzgerald & Co. ("Cantor") and Stifel, Nicolaus & Company, Incorporated, ("Stifel"), under which the Company may, from time to time, offer and sell common stock having an aggregate offering value of up to $150.0 million, referred to as our "at-the-market" offering with Cantor and Stifel. As of March 31, 2021, no such shares of the Company's common stock had been offered or sold pursuant to this "at-the-market" program with Cantor and Stifel. The Company may terminate this at-the-market program at any time, pursuant to its terms.

Follow-on public offering

In November 2020, the Company completed an underwritten public offering and issued and sold 2,875,000 shares of common stock at a public offering price of $39.00 per share, which included 375,000 shares sold upon full exercise of the underwriters' option to purchase additional shares of common stock, resulting in net proceeds of $104.9 million after deducting underwriting discounts and commissions and offering expenses

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 condensed consolidated financial statements 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 these unaudited condensed consolidated financial statements.

2. Summary of significant accounting policies and recent accounting pronouncements

Basis of presentation and consolidation

The accompanying unaudited condensed consolidated financial statements have been prepared in accordance with U.S. generally accepted accounting principles ("GAAP") and include the accounts of the Company and its wholly-owned subsidiary. Any reference in these notes to applicable guidance is meant to refer to GAAP as found in the Accounting Standards Codification ("ASC") and Accounting Standards Update ("ASU") of the Financial Accounting Standards Board ("FASB"). All intercompany transactions between and among its consolidated subsidiary have been eliminated.

Unaudited interim financial information

The accompanying interim unaudited condensed consolidated financial statements and related disclosures are unaudited and have been prepared in accordance with GAAP for interim financial information and the instructions to Form 10-Q and Article 10 of Regulation S-X. Accordingly, they do not include all the information and footnotes required by GAAP for complete financial statements and should be read in conjunction with the Company's consolidated financial statements and related footnotes as of and for the year ended December 31, 2020, which was filed with the SEC on March 9, 2021. The Company's financial information as of March 31, 2021, and for the three months ended March 31, 2021 and 2020 is unaudited, but in the opinion of management, all adjustments, consisting only of normal recurring adjustments, considered necessary for a fair presentation of the financial position, results of operations and cash flows at the dates and for the periods presented of the results of these interim periods have been included. The balance sheet information as of December 31, 2020 was derived from audited financial statements. The results of the Company's operations for any interim period are not necessarily indicative of the results that may be expected for any other interim period or for a full fiscal year.

Use of estimates

The preparation of unaudited condensed 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 those 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 and money market accounts.

Restricted cash

At March 31, 2021, restricted cash consisted of money market accounts collateralizing letters of credit issued as security deposits in connection with the Company's leases of its corporate facilities.

The following table reconciles cash and cash equivalents and restricted cash per the condensed consolidated balance sheets to the condensed consolidated statements of cash flows (in thousands):

	As of March 31,	
	2021	2020
Cash and cash equivalents	$ 267,514	$ 211,288
Restricted cash - short-term	$ 147	$ —
Restricted cash	$ 58	$ 205
Total cash, cash equivalents and restricted cash	$ 267,719	$ 211,493

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 the condensed consolidated statement of stockholders' equity as a reduction of additional paid-in capital.

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 condensed 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 (1) the last day of its first fiscal year (a) in which the Company has total annual gross revenues of at least $1.07 billion, or (b) in which the Company is deemed to be a large accelerated filer, which means the market value of its common stock that is held by non-affiliates exceeds $700.0 million as of the prior June 30th, (2) the date on which it has issued more than $1.0 billion in non-convertible debt securities during the prior three-year period and (3) December 31, 2024.

The Company is also a "smaller reporting company," meaning that, the market value of its stock held by non-affiliates is less than $700 million and our 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 February 2016, the FASB issued Accounting Standards Update ("ASU") No. 2016-02, *Leases* (Topic 842). This standard established a right-of-use model that requires all lessees to recognize right-of-use assets and lease liabilities on their balance sheet that arise from leases as well as provide disclosures with respect to certain qualitative and quantitative information related to a company's leasing arrangements. The Company adopted Topic 842 on January 1, 2020 using the modified retrospective approach and elected to apply the transition method that allows companies to continue applying guidance under the lease standard in effect at that time in the comparative period financial statements and recognize a cumulative-effect adjustment to the balance sheet on the date of adoption. The Company elected the package of practical expedients to not reassess its prior conclusions about lease identification, lease classification and indirect costs and to not separate lease and non-lease components.

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 were 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. Early adoption is permitted for all entities, including adoption in an interim period. The Company adopted Part 1 of this standard on January 1, 2020 and the adoption of this update did not have a material impact 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 is effective for all entities, for fiscal years beginning after December 15, 2019 with early adoption permitted. The Company adopted this standard on January 1, 2020 and the adoption of this update did not have a material impact on its consolidated financial statements.

In December 2019, the FASB issued ASU 2019-12, Income Taxes (Topic 740): Simplifying the Accounting for Income Taxes. This guidance removes certain exceptions for recognizing deferred taxes for investments, performing intraperiod allocation and calculating income taxes in interim periods. It also adds guidance to reduce complexity in certain areas, including recognizing deferred taxes for tax goodwill and allocating taxes to members of a consolidated group. This ASU is effective for interim and annual periods beginning after December 15, 2020, and early adoption is permitted. The Company adopted this standard on January 1, 2021 and the adoption of this update did not have a material impact on its consolidated financial statements.

3. Fair value measurements

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 (in thousands):

	Fair value measurements as of March 31, 2021			
	Level 1	Level 2	Level 3	Total
Cash equivalents:				
Money market funds	$ 267,514	$ —	$ —	$ 267,514
Total	$ 267,514	$ —	$ —	$ 267,514

	Fair value measurements as of December 31, 2020			
	Level 1	Level 2	Level 3	Total
Cash equivalents:				
Money market funds	$ 287,308	$ —	$ —	$ 287,308
Total	$ 287,308	$ —	$ —	$ 287,308

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 condensed consolidated balance sheets as of March 31, 2021 and December 31, 2020.

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 condensed 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 (in thousands):

	March 31, 2021	December 31, 2020
Accrued employee compensation costs	$ 1,079	$ 4,123
Accrued professional	694	593
Accrued research and development costs	1,588	3,689
Accrued Other	87	82
Other Current Liabilities	1,166	1,443
	$ 4,614	$ 9,930

5. Commitments and contingencies

Operating lease

In February 2016, the FASB issued ASU No. 2016-02, *Leases* (Topic 842). This standard established a right-of-use model that requires all lessees to recognize right-of-use assets and lease liabilities on their balance sheet that arise from leases as well as provide disclosures with respect to certain qualitative and quantitative information related to a company's leasing arrangements. The Company adopted Topic 842 on January 1, 2020 using the modified retrospective approach and elected to apply the transition method that allows companies to continue applying guidance under the lease standard in effect at that time in the comparative period financial statements and recognize a cumulative-effect adjustment to the balance sheet on the date of adoption. The Company elected the package of practical expedients to not reassess its prior conclusions about lease identification, lease classification and indirect costs and to not separate lease and non-lease components.

Upon adoption of Topic 842 on January 1, 2020, the Company recorded right-of-use assets of $2.2 million, operating lease liabilities of $2.2 million and the elimination of deferred rent of $0.03 million. Adoption of the standard did not result in the Company recording a cumulative effect adjustment.

The Company determines whether an arrangement is a lease at inception. The Company accounts for a lease when it has the right to control the leased asset for a period of time while obtaining substantially all of the assets' economic benefits. The Company determined that it held operating leases for office and laboratory space as of January 1, 2020. Operating lease right-of-use assets and operating lease liabilities are recognized based on the present value of the future minimum lease payments over the lease term at the

lease commencement date. The discount rate used to determine the present value of the lease payments is the Company's incremental borrowing rate based on the information available at lease inception, as the Company did not have information to determine the rate implicit in the leases. Lease expense for operating leases is recognized on a straight-line basis over the reasonably assured lease term based on the total lease payments (which include initial direct costs and lease incentives). The expense is included in operating expenses in the condensed consolidated statements of operations. The Company's lease agreements also contain variable payments, primarily maintenance-related costs, which are expensed as incurred and not included in the measurement of the right-of-use assets and lease liabilities.

In August 2018, the Company entered into an agreement to lease 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, the Company entered into an agreement to lease 2,485 square feet of space for an initial term of three years. The lease includes one renewal option for an additional two years, however, any time after the initial term the landlord may relocate the Company from the premises to a space reasonably comparable in size and utility. As the Company does not have the right to control the use of the identified asset after the initial term, the renewal option was excluded from the lease liability calculation. Lease terms commence at $0.2 million per annum, with 2.5% annual base rent increases plus operating expenses, real estate taxes, utilities and janitorial fees. The lease commencement date was May 1, 2019.

Future minimum lease payments under non-cancellable leases as of March 31, 2021 are as follows (in thousands):

2021	808
2022	81
Total lease payments	889
Less imputed interest	(21)
Present value of lease liabilities	$ 868

Future minimum lease payments under non-cancellable leases as of December 31, 2020 are as follows (in thousands):

2021	1,102
2022	81
Total lease payments	1,183
Less imputed interest	(37)
Present value of lease liabilities	$ 1,146

Lease balances as of March 31, 2021 are as follows (in thousands):

Operating right-of-use assets	$ 844
Current Portion of operating lease liabilities	$ 848
Non-current portion of operating lease liabilities	20
Total operating lease liabilities	$ 868

The weighted average remaining lease term and weighted average discount rate of our operating leases as of March 31, 2021 are as follows:

Weighted average remaining lease term in years	0.8
Weighted average discount rate	7.01%

Consulting agreement

In October 2014, the Company entered into a consulting agreement with a member of the Company's 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. The Company recognized expenses of $0.02 million in the three-month period ending March 31, 2020, for such consulting services. The initial term of this agreement was five years and the parties mutually agreed not to extend the consulting agreement in April 2020.

Scientific Advisory Board Agreement

In June 2020, the Company entered into a scientific advisory board agreement with a member of the Company's board of directors, who is also an employee of CSHL, to provide scientific advisory services related to the Company's Targeted Augmentation of Nuclear Gene Output ("TANGO") antisense oligonucleotide technology and other antisense oligonucleotide technologies, as well as current and future therapeutic targets and programs. The Company recognized expense of $0.01 million in the three-month period ended March 31, 2021, for such scientific advisory services. The term of this agreement is 12 months.

License and research agreements

In July 2015, the Company entered into a worldwide license agreement (the "CSHL Agreement"), with CSHL, with respect to 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 $0.07 million. 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 maximum of twenty percent of the sublicense revenue to CSHL, which may be reduced to a mid-teens 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 $0.01 million, which amount is creditable against any owed royalty or milestone payments. The maximum aggregate potential milestone payments payable total approximately $0.9 million. 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, 2021 and 2020.

In April 2016, the Company entered into an exclusive, worldwide license agreement with the University of Southampton, (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 0.06 million pounds sterling (approximately $0.08 million 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 totaled approximately 0.4 million pounds sterling (approximately $0.6 million as of March 31, 2021). As of March 31, 2021, and December 31, 2020, the Company had recorded no liabilities under the Southampton Agreement.

6. Equity incentive plans

In June 2019, the Company's board of directors and stockholders approved the 2019 Equity Incentive Plan (the "2019 Plan") which became effective on June 17, 2019 and replaced the Company's 2014 Equity Incentive Plan (the "2014 Plan"). In addition to the shares of common stock reserved for future issuance under the 2014 Plan that were added to the 2019 Plan upon its effective date, the Company initially reserved 2,200,000 shares of common stock for issuance under the 2019 Plan. The number of shares reserved for issuance under the Company's 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 the Company's common stock as of the immediately preceding December 31, or a lesser number as may be determined by the Company's board of directors.

As of March 31, 2021, there were no shares available for future issuance under the 2014 Plan and 2,612,512 shares were available under the 2019 Plan.

During the three-months ended March 31, 2021, the Company granted options to purchase 1,022,547 shares of common stock to certain of its employees. 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.

Stock-based compensation

As of March 31, 2021, there was $53.6 million of unrecognized compensation cost related to unvested stock-based compensation arrangements granted under the 2014 and 2019 Plans. The compensation is expected to be recognized over a weighted average period of 3.53 years as of March 31, 2021.

Stock-based compensation expense recorded as research and development and general and administrative expenses in the accompanying condensed consolidated statements of operations and comprehensive loss is as follows (in thousands):

	Three Months Ended March 31,	
	2021	2020
Research and development	$ 1,034	$ 204
General and administrative	1,664	550
	$ 2,698	$ 754

2019 Employee stock purchase plan

In June 2019, the Company adopted the 2019 Employee Stock Purchase Plan (the "ESPP"), which became effective on June 18, 2019. The Company initially reserved 315,000 shares of common stock for sale under the ESPP. At March 31, 2021, the Company had 654,579 shares available for issuance under the plan. The average grant date fair value per share under the plan was $58.07 for 2021. The total ESPP stock-based compensation expense for the three months ended March 31, 2021 was $0.08 million. The number of shares reserved for issuance under the ESPP will increase automatically on January 1st of each of the first ten calendar years following the first offering date by the number of shares equal to the lesser of 1% of the total outstanding shares of the Company's common stock as of the immediately preceding December 31 or a lower amount determined by the Company's board of directors. The aggregate number of shares issued over the term of the ESPP will not exceed 3,150,000 shares of the Company's common stock.

7. Net loss per share

The following table summarizes the computation of basic and diluted net loss per share of the Company (in thousands except share and per share amounts):

	Three Months Ended March 31,	
	2021	2020
Numerator:		
Net loss	$ (16,793)	$ (11,039)
Denominator:		
Weighted-average number of common shares, basic and diluted	36,643,205	32,897,395
Net loss per share, basic and diluted	$ (0.46)	$ (0.34)

The Company's potential dilutive securities, which include common stock options and ESPP purchase rights, 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 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 indicated because including them would have had an anti-dilutive effect:

	March 31,	
	2021	2020
Outstanding options to purchase common stock	5,375,811	4,992,251
	5,375,811	4,992,251

8. Income taxes

The Company did not record an income tax benefit in its condensed consolidated statement of operations and comprehensive loss for the three months ended March 31, 2021 and 2020 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 $37.2 million at December 31, 2020. The Company has provided a valuation allowance for the full amount of its net deferred tax assets and liabilities as of March 31, 2021 and December 31, 2020, 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.

On March 27, 2020, the Coronavirus Aid, Relief, and Economic Security Act ("CARES Act") was enacted in response to the COVID-19 pandemic. The CARES Act, among other things, permits net operating loss ("NOL") carryovers and carrybacks to offset 100% of taxable income for taxable years beginning before 2021. In addition, the CARES Act allows NOLs incurred in 2018, 2019, and 2020 to be carried back to each of the five preceding taxable years to generate a refund of previously paid income taxes. The Company has evaluated the impact of the CARES Act, and at present, the Company does not expect that the NOL carryback provision of the CARES Act would result in a cash benefit to us.

The Company did not record any amounts for unrecognized tax benefits as of March 31, 2021 or December 31, 2020.

9. Subsequent events

The Company has evaluated subsequent events through the issuance date of these interim unaudited condensed consolidated financial statements.

Item 2. 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 "Risk Factors" and our interim condensed consolidated financial statements and related notes appearing elsewhere in this Quarterly Report. Some of the information contained in this discussion and analysis or set forth elsewhere in this Quarterly Report, 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 sections entitled "Special Note Regarding Forward-Looking Statements" and "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 a biotechnology company dedicated to addressing the underlying cause of severe diseases by up-regulating protein expression with RNA-based medicines. We are developing novel antisense oligonucleotide ("ASO"), medicines that target ribonucleic acid ("RNA"), and modulate precursor-messenger RNA splicing to upregulate protein expression where needed and with appropriate specificity to near normal levels. We utilize our proprietary RNA therapeutics platform, Targeted Augmentation of Nuclear Gene Output ("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, eye, kidney and liver.

We were incorporated in June 2014. In July 2015 and April 2016, we entered into worldwide license agreements with Cold Spring Harbor Laboratory ("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 June 21, 2019, our operations have been financed primarily from the sale of convertible notes payable and our convertible preferred stock.

In June 2019, we completed an initial public offering ("IPO") and issued and sold 9,074,776 shares of common stock at a public offering price of $18.00 per share, which included 1,183,666 shares sold upon full exercise of the underwriters' option to purchase additional shares of common stock resulting in net proceeds of $149.4 million after deducting underwriting discounts and commissions and offering costs.

In July 2020, we filed a universal shelf registration statement on Form S-3 (the "Registration Statement") with the SEC. The Registration Statement was declared effective by the SEC in July 2020, and covers the offering, issuance and sale by us of up to a maximum aggregate offering price of $400,000,000 of our common stock, preferred stock, debt securities, warrants to purchase our common stock, preferred stock or debt securities, subscription rights to purchase our common stock, preferred stock or debt securities and/or units consisting of some or all of these securities. In July 2020, we entered into an "at-the-market" program and sales agreement with Cantor Fitzgerald & Co., or Cantor, and Stifel, Nicolaus & Company, Incorporated, or Stifel, under which we may, from time to time, offer and sell common stock having an aggregate offering value of up to $150.0 million, referred to as our "at-the-market" offering with Cantor and Stifel. As of March 31, 2021, no such shares of our common stock had been offered or sold pursuant to this "at-the-market" program with Cantor and Stifel. We may terminate this at-the-market program at any time, pursuant to its terms.

In November 2020, we completed an underwritten public offering, of our common stock and issued and sold 2,875,000 shares of common stock at a public offering price of $39.00 per share, which included 375,000 shares sold upon full exercise of the underwriters' option to purchase additional shares of common stock resulting in net proceeds of $104.9 million after deducting underwriting discounts and commissions and estimated offering expenses.

As of March 31, 2021 and December 31, 2020 we had $267.7 million and $287.5 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 $16.8 million and $11.0 million for the three months ended March 31, 2021 and 2020, respectively. As of March 31, 2021, we had an accumulated deficit of $127.1 million and as of December 31, 2020 we had an accumulated deficit of $110.3 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 Securities and Exchange Commission ("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 our existing cash, cash equivalents and restricted cash as of March 31, 2021, will enable us to fund our operating expenses and capital expenditure requirements into 2024. 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.

Business Update Regarding COVID-19

The current COVID-19 pandemic has presented a substantial public health and economic challenge around the world and is affecting our employees, patients, communities and business operations, as well as the U.S. economy and financial markets. The full extent to which the COVID-19 pandemic will directly or indirectly impact our business, results of operations, liquidity and financial condition will depend on future developments that are highly uncertain and cannot be accurately predicted, including new information that may emerge concerning COVID-19, the actions taken to contain it or treat its impact and the economic impact on local, regional, national and international markets.

To date, our third party contract research organizations ("CROs"), contract manufacturing organizations ("CMOs"), and other third party vendors have been able to continue to provide services and supply reagents, materials, and products and currently do not anticipate any significant disruption in services or interruptions in supply. We have pursued mitigation strategies to keep key research activities on track. Our third-party CMOs continue to operate their manufacturing facilities at or near normal levels. While we currently do not anticipate any significant interruptions in our manufacturing processes, it is possible that the COVID-19 pandemic and response efforts may have an impact in the future on our third-party suppliers and CMO's ability to manufacture reagents, materials, or products that we need to use in our research and clinical trial. However, we are continuing to assess the potential impact of the COVID-19 pandemic on our business and operations, including our expenses, our observational study, our clinical trials, and our ability to hire and retain employees.

Our ability to continue our clinical trials and our observational study may be adversely affected, directly or indirectly, by the COVID-19 pandemic. Currently we are monitoring patient enrollment and participation in our clinical trials and our observational study, including the duration and degree to which we may see declines in enrollment, delays in conducting in-person follow-ups, and disruptions in our ability to monitor patients due to hospitals closing sites or diverting the resources that are necessary to conduct our clinical trials and our observational study. While we continue to enroll and dose patients in our clinical trials at sites across the United States, we expect that COVID-19 precautions may directly or indirectly impact the timeline for some of our clinical trial activities due to hospitals closing sites and/or diverting the resources that are necessary to conduct clinical trials. We are pursuing approaches to help mitigate the impact to our clinical trials. Currently we have not experienced any delays in our clinical trials.

The COVID-19 pandemic has caused us to modify our business practices (including but not limited to curtailing or modifying employee travel, moving to partial remote work, and cancelling physical participation in meetings, events and conferences) and we may take further actions as may be required by government authorities or that we determine are in the best interests of our employees, patients and business partners.

Our office-based employees have been working from home since early March 2020, while ensuring essential staffing levels in our operations remain in place, including maintaining key personnel in our laboratories.

For additional information on the various risks posed by the COVID-19 pandemic, please read Item 1A. Risk Factors included in this report.

Program Update

Dravet Syndrome Program - STK-001

We designed our lead product candidate, STK-001, to treat Dravet syndrome, a severe and progressive genetic epilepsy. This program draws on a well-defined patient population based on routine genetic testing and learnings from drugs approved for the treatment of Dravet syndrome to inform the clinical and regulatory pathways for STK-001.

We submitted an investigational new drug application ("IND") for STK-001 to the U.S. Food and Drug Administration (the "FDA") in late 2019. In August 2020, we dosed the first patient with STK-001 in the single ascending dose portion of the MONARCH Phase 1/2a Study at the 10mg dose level.

The MONARCH Study is designed to evaluate single and multiple ascending dose levels of STK-001 in children and adolescents with Dravet syndrome. Patients are eligible for the trial if they are between the ages of 2 to 18, 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 1,700 *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 primary objectives are the assessment of the safety and tolerability of STK-001, as well as to characterize human pharmacokinetics. A secondary objective is to assess the efficacy as an adjunctive antiepileptic treatment with respect to the percentage change from baseline in convulsive seizure frequency over a 12-week treatment period. We are measuring non-seizure aspects of the disease, such as quality of life as secondary endpoints. These endpoints as well as other exploratory endpoints will be informed based on our two-year observational study ("BUTTERFLY"). Enrollment in BUTTERFLY is complete and the study is ongoing. BUTTERFLY is designed to evaluate seizure frequency and non-seizure comorbidities associated with the disease, including motor and speech impairment, intellectual and developmental disabilities, behavioral deficits and abnormal sleep patterns. Data from the study will support clinical development plans for STK-001.

In March 2020, we announced the FDA had placed a partial clinical hold on doses of STK-001 above 20mg in the MONARCH Study, pending additional preclinical testing to determine the safety profile of doses higher than the current no observed adverse effect level ("NOAEL"). When intrathecal doses above the NOAEL were administered to non-human primates ("NHPs") acute, transient adverse hind limb paresis was observed. This finding is known to occur following intrathecal delivery of ASOs to NHPs and is not known to translate to the human experience. When extremely high dose levels were administered, acute convulsions were observed immediately following STK-001 administration. The dose levels were well above the range of corresponding human doses that would ever be administered in the clinic and were delivered in a formulation that was at a higher concentration than would be administered in the clinic. There is no apparent correlation of these acute adverse events with the mechanism of action of STK-001.

Following interactions with the FDA, in October 2020, we announced that the FDA agreed to allow us to add an additional higher dose level to the SAD portion of the study such that a total of three dose levels will be evaluated: 10 mg, 20 mg, and 30mg. Dosing above 30mg in this study remains on FDA partial clinical hold.

In addition, a multiple ascending dose ("MAD") portion was added to the MONARCH Study based on preclinical repeat-dose toxicology data, which were reviewed by the FDA. There were no adverse effects observed in the NHP repeat dose study.

Enrollment and dosing in the 10mg, 20mg and 30mg single ascending dose portion of the study are now complete. We expect to announce initial safety and pharmacokinetic data from the SAD portion of the MONARCH Study in the third quarter of 2021. In February 2021, the first patient in the MAD portion of the study was dosed with STK-001 at the 20mg level.

In January 2021, we initiated enrollment and dosing in our SWALLOWTAIL Open Label Extension (OLE) study of STK-001 for children and adolescents with Dravet syndrome. SWALLOWTAIL is designed to evaluate the long-term safety and tolerability of repeated doses of STK-001 in patients with Dravet syndrome who previously participated in studies of STK-001. In this study, patients will initially be administered the same dose level they received in the previous study and will receive a maximum of three doses. One dose will be administered every four months.

In March 2021, we announced the authorization of our Clinical Trial Application (CTA) by the United Kingdom Medicines and Healthcare products Regulatory Agency (MHRA) to initiate a Phase 1/2a study (ADMIRAL) of STK-001 for the treatment of Dravet syndrome. ADMIRAL is an open-label, multi-center, Phase 1/2a study designed to assess the safety and tolerability of multiple doses of up to 70mg of STK-001, as well as to characterize human pharmacokinetics. Secondary endpoints include change in seizure frequency and quality of life measures. The study is expected to enroll more than 20 children and adolescents with Dravet syndrome across multiple clinical sites in the United Kingdom. Patient enrollment and dosing are expected to start in the second half of 2021.

While we continue working to minimize any potential delay to continued clinical testing of STK-001, we expect that the COVID-19 pandemic may directly or indirectly impact the timeline. Our ability to continue our clinical trials may be adversely affected, directly or indirectly, by the COVID-19 pandemic. For example, although our Phase 1/2a study of STK-001 for the treatment of Dravet syndrome has not been delayed, we plan to have clinical trial sites in various parts of the United States and United Kingdom, some of

which have had high incident rates of COVID-19 patients. Restrictions on travel and/or transport of clinical materials, as well as diversion of hospital staff and resources to COVID-19 infected patients, could disrupt trial operations as well as recruitment, possibly resulting in a slowdown in enrollment and/or deviations from or disruptions in key clinical trial activities, such as clinical trial site monitoring. These challenges may lead to difficulties in meeting protocol-specified procedures.

We have not yet discussed with regulatory authorities the evidence necessary for approval of STK-001. However, in addition to requesting Fast Track Designation, if we see evidence of clinical efficacy, then we would plan to meet with regulatory authorities to discuss expedited regulatory pathways, such as Breakthrough Therapy Designation in the United States.

Autosomal Dominant Optic Atrophy Program

In November 2020, we announced the nomination of OPA1 as our next target for preclinical development to treat Autosomal Dominant Optic Atrophy ("ADOA"). ADOA is the most common inherited optic nerve disorder seen in clinical practice and causes progressive and irreversible vision loss in both eyes starting in the first decade of life. Many children progress to blindness. The disease affects one in 30,000 people globally with a higher incidence of approximately one in 10,000 in Denmark due to a founder effect. 65%-90% of cases are caused by mutations in one allele of the OPA1 gene, most of which lead to a haploinsufficiency resulting in 50% of OPA1 protein expression and disease manifestation.. More than 400 different OPA1 mutations have been reported in people diagnosed with ADOA. Most mutations result in a severe decrease – up to 50% – of the normal amount of the OPA1 protein. There is no strong genotype-phenotype correlation.

OPA1 is a dynamin-related GTPase that plays a key role in maintaining mitochondria structure and dynamics. The OPA1 protein is imported into the mitochondria and is the crucial molecule that mediates inner mitochondria membrane fusion and cristae morphogenesis and is critical for oxidative phosphorylation and ATP synthesis. Insufficient OPA1 activity causes mitochondria dysfunction with consequent insufficient ATP production, excess reactive oxygen species production and eventual cell death. High energy demanding cells such as neurons and cardiomyocytes are particularly susceptible to mitochondria dysfunction, and retinal ganglion cells ("RGCs") are a neuronal cell type most susceptible to loss of OPA1 protein as evidenced by RGC death in ADOA caused by OPA1 haploinsufficiency.

A clinical diagnosis of dominant optic atrophy is made when a patient meets some or all of the following criteria: pathogenic variant of the OPA1 gene identified in the patient or a family member; reduced visual acuity; temporal disc pallor; visual field defect; color vision defect (acquired blue-yellow loss); thinning of retinal nerve fiber layer and abnormal visual evoked potentials. Clinical findings are based on: intraocular pressure measurement; visual field assessment; color discrimination; dilated slit lamp biomicroscopy; optical coherence tomography; or visual electrophysiology. Patients suspected of dominant optic atrophy are recommended to receive genetic testing to confirm the clinical diagnosis, help identify other family members who are affected and ensure patients avoid stressors that could increase disease progression (e.g. smoking, alcohol). The prognosis for many patients with dominant optic atrophy is poor and the rate of visual loss can be difficult to predict given significant inter- and intra-familial variability.

There are currently no available treatments for dominant optic atrophy. Because ADOA causes deterioration of the optic nerves, corrective aids such as glasses or contacts do not help to improve vision lost to the disease. Supportive services and low-vision aids are offered for patients with severely decreased visual acuity. Our ASOs are designed to target the underlying cause of ADOA, which is OPA1 haploinsufficiency, by decreasing a non-productive mRNA splicing event in the OPA1 gene to increase productive OPA1 mRNA and OPA1 protein in the retinal ganglion cells. Lead optimization to identify a clinical candidate is currently underway, we hope to identify a candidate by year end 2021.

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 consists 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 (in thousands):

	Three Months Ended March 31,	
	2021	2020
OPA1	$ 78	$ —
STK-001	3,124	2,508
Non-program specific and unallocated research and development expenses	6,711	4,707
Total research and development expenses	$ 9,913	$ 7,215

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, which may be directly or indirectly impacted by the COVID-19 pandemic, 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, 2021 and 2020

The following table sets forth our results of operations:

	Three Months Ended March 31,	
	2021	2020
	(in thousands)	
Condensed Consolidated statements of operations:		
Revenue	$ —	$ —
Operating expenses:		
Research and development	9,913	7,215
General and administrative	6,914	4,520
Total operating expenses	$ 16,827	$ 11,735
Loss from operations	$ (16,827)	$ (11,735)
Other income:		
Interest income	18	674
Other income (expense), net	16	22
Total other income	$ 34	$ 696
Net loss	$ (16,793)	$ (11,039)

Research and development expenses

Research and development expenses were $9.9 million for the three months ended March 31, 2021 as compared to $7.2 million for the three months ended March 31, 2020, an increase of $2.7 million. The table below summarizes our research and development expenses (in thousands):

	Three Months Ended March 31,	
	2021	2020
OPA1	$ 78	$ —
STK-001	3,124	2,508
Personnel-related expenses	3,601	2,394
Third-party services	443	402
Scientific consulting	99	351
Facilities and other research and development expenses	2,568	1,560
Total research and development expenses	$ 9,913	$ 7,215

The increase in research and development expenses were primarily attributable to an increase of $1.2 million in personnel costs resulting from an increase in headcount, an increase of $1.0 million in facilities and other costs, an increase of $0.6 million in expenses related to our STK-001 program and $0.1 million related to our OPA1 program, which comprised of third-party services and scientific consulting fees, partially offset by a decrease of $0.2 million in non-project specific consulting and third-party services, materials and other costs. The increases in expense reflect the accelerating pace of research and development activities and the increases in personnel, facilities and, third party services to support those activities.

General and administrative expenses

General and administrative expenses were $6.9 million for the three months ended March 31, 2021 as compared to $4.5 million for the three months ended March 31, 2020, an increase of $2.4 million.

The increase in general and administrative expenses were primarily attributable to an increase of $0.5 million in personnel costs resulting from an increase in headcount, and an increase of $1.9 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 and expenses including stock compensation expense.

Other income

The change in our other income for the three months ended March 31, 2021 as compared to the three months ended March 31, 2020 primarily reflects an increase in cash balances offset by a decrease in market interest rates.

Liquidity and capital resources

Since our inception through March 31, 2021, our operations have been primarily financed by net proceeds of $385.3 million from the sale of convertible notes payable and our convertible preferred stock as well as our IPO and follow-on offering.

As of March 31, 2021, we had $267.7 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, 2021, we had an accumulated deficit of $127.1 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.

Based upon our current operating plan, we believe that our existing cash, cash equivalents and restricted cash as of March 31, 2021 will enable us to fund our operating expenses and capital expenditure requirements into 2024. 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 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, and the full extent to which the COVID-19 pandemic will directly or indirectly impact our business, 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,	
	2021	2020
	(in thousands)	
Net cash (used in) provided by:		
Operating activities	$ (20,136)	$ (10,771)
Investing activities	(204)	(610)
Financing activities	546	198
Net increase (decrease) in cash, cash equivalents and restricted cash	$ (19,794)	$ (11,183)

Operating activities

During the three months ended March 31, 2021, cash used in operating activities was $20.1 million and was primarily attributable to a net loss of $16.8 million, partially offset by non-cash charges of $3.2 million for share-based compensation, depreciation, and reduction in the carrying amount of right of use assets, and a net change of $6.6 million in our net operating assets and liabilities.

During the three months ended March 31, 2020, cash used in operating activities was $10.8 million and was primarily attributable to a net loss of $11.0 million, partially offset by non-cash charges of $1.2 million for share-based compensation, depreciation, and reduction in the carrying amount of right of use assets, and a net change of $1.0 million in our net operating assets and liabilities.

Investing activities

Our investing activities during the three months ended March 31, 2021 and 2020 have consisted principally of purchases of property and equipment.

Financing activities

Our financing activities during the three months ended March 31, 2021 consisted of $0.4 million from the exercise of stock options and $0.2 million in proceeds from our Employee Stock Purchase Plan.

Our financing activities during the three months ended March 31, 2020 consisted primarily of $0.2 million from the exercise of stock options.

Contractual obligations and commitments

The following table summarizes our contractual obligations as of March 31, 2021 and the effects that such obligations are expected to have on our liquidity and cash flows in future fiscal periods:

	Payments Due by Fiscal Period				
	Total	Less Than 1 Year	1 to 3 Years	4 to 5 Years	More than 5 Years
	(in thousands)				
Operating lease obligations	$ 889	$ 808	$ 81	$ —	$ —
Total	$ 889	$ 808	$ 81	$ —	$ —

In August 2018, we entered into an agreement to lease 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. The lease commencement date was May 1, 2019.

Commitments

Our commitments primarily consist of obligations under our agreements with CSHL and the University of Southampton. As of March 31, 2021, 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 *Note 5—Commitments and Contingencies* of the notes to our condensed consolidated financial statements included elsewhere in this Quarterly Report on Form 10-Q.

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 condensed consolidated financial statements, which have been prepared in accordance with generally accepted accounting principles ("GAAP"). The preparation of these condensed consolidated 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 condensed 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.

There have been no significant changes in our critical accounting policies and estimates as compared to the critical accounting policies and estimates disclosed in the section titled "Management's Discussion and Analysis of Financial Condition and Operations" included in our Annual Report on Form 10-K filed with the SEC on March 9, 2021.

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 (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 condensed 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 (1) the last day of our first fiscal year (a) in which we have total annual gross revenues of at least $1.07 billion, or (b) in which 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 $700.0 million as of the prior June 30th, (2) the date on which we have issued more than $1.0 billion in non-convertible debt securities during the prior three-year period and (3) December 31, 2024.

We are also a "smaller reporting company," meaning that the market value of our stock held by non-affiliates was less than $700 million and its annual revenue is less than $100 million during the most recently completed fiscal year. We may continue to be a smaller reporting company as long as 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 and recently issued accounting pronouncements

See *Note 2—Summary of significant accounting policies and recent accounting pronouncements* of the notes to our unaudited condensed consolidated financial statements included elsewhere in this Quarterly Report on Form 10-Q for more information.

Item 3. 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 $267.7 million as of March 31, 2021 and $287.5 million as of December 31, 2020. 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. A hypothetical 100 basis point change in interest rates would affect the fair market value of our cash equivalents by approximately $2.7 million.

Item 4. Controls and Procedures.

Conclusion Regarding the Effectiveness of Disclosure Controls and Procedures

Under the supervision and with the participation of our management, including our Chief Financial Officer and Chief Executive Officer, we evaluated the effectiveness of the design and operation of our disclosure controls and procedures (as defined in Rules 13a-15(e) and 15d-15(e) under the Securities Exchange Act of 1934 (the "Exchange Act") as of March 31, 2021. The term "disclosure controls and procedures," as defined in Rules 13a-15(e) and 15d-15(e) under the Exchange Act, means controls and other procedures of a company that are designed to ensure that information required to be disclosed by a company in the reports that it files or submits under the Exchange Act is recorded, processed, summarized and reported within the time periods specified in the SEC's rules and forms. Disclosure controls and procedures include, without limitation, controls and procedures designed to ensure that information required to be disclosed by a company in the reports that it files or submits under the Exchange Act is accumulated and communicated to the company's management, including its principal executive and principal financial officers, as appropriate, to allow timely decisions regarding required disclosure. Management recognizes that any controls and procedures, no matter how well designed and operated, can provide only reasonable assurance of achieving their objectives and management necessarily applies its judgment in evaluating the cost-benefit relationship of possible controls and procedures. Based on our management's evaluation (with the participation of our Chief Executive Officer and our Chief Financial Officer), as of the end of the period covered by this report, our Chief Executive Officer and our Chief Financial Officer have concluded that our disclosure controls and procedures were effective at the reasonable assurance level.

Changes in Internal Control over Financial Reporting

There has been no change in our internal control over financial reporting (as defined in Rules 13a-15(f) and 15d-15(f) of the Exchange Act) during the period covered by this report that has materially affected, or is reasonably likely to materially affect, our internal control over financial reporting. We have not experienced any material impact to our internal controls over financial reporting despite the fact that most of our employees are working remotely due to the COVID-19 pandemic. We are continually monitoring and assessing the COVID-19 situation on our internal controls to minimize the impact on their design and operating effectiveness.

Inherent Limitations on Effectiveness of Controls

Internal control over financial reporting may not prevent or detect all errors and all fraud. Any controls and procedures, no matter how well designed and operated, can provide only reasonable assurance of achieving the desired control objective and management necessarily applies its judgment in evaluating the cost-benefit relationship of possible controls and procedures.

PART II – OTHER INFORMATION

Item 1. 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.

Item 1A. Risk Factors.

Summary of Risk Factors

An investment in our common stock involves various risks, and prospective investors are urged to carefully consider the matters discussed in the section titled “Risk Factors” prior to making an investment in our common stock. These risks include, but are not limited to, 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.
- Success in early preclinical studies or clinical trials may not be indicative of results obtained in later preclinical studies and clinical trials, including in our Dravet syndrome program or our ADOA program.
- 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.
- The ongoing COVID-19 pandemic may, directly or indirectly, adversely affect our business, results of operations and financial condition.
- 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.
- If clinical trials of STK-001, the product candidate we select to treat ADOA or any other product candidate that we develop fail to demonstrate safety and efficacy to the satisfaction of FDA or foreign regulatory authorities or do not otherwise produce favorable results, we may incur additional costs or experience delays in completing, or ultimately may be unable to complete, the development and commercialization of such product candidate.
- 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 failure to obtain regulatory approval in international jurisdictions would prevent us from marketing our product candidates outside the United States.
- STK-001 or our future product candidates, including the product candidate we select to treat ADOA, 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.
- A Fast Track Designation by the FDA, even if granted for 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.
- A Breakthrough Therapy Designation by the FDA for STK-001 or our future product candidates, including the product candidate we select to treat ADOA, 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.
- 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.
- The commercial success of our product candidates, including STK-001 and the product candidate we select to treat ADOA will depend upon their degree of market acceptance by providers, patients, patient advocacy groups, third-party payors and the general medical community.
- 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.
- Current and potential future healthcare reforms may adversely impact pricing, insurance coverage and reimbursement status of newly approved products.

- 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 expect that we will need to raise additional funding before we can expect to become profitable from any potential future sales of STK-001, the product candidate we select to treat ADOA 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.
- 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.
- 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.
- The market price of our stock may be volatile, 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 our Targeted Augmentation of Nuclear Gene Output ("TANGO") technology and our current lead product candidate, STK-001, for the treatment of Dravet syndrome. We submitted an investigational new drug application ("IND") for STK-001 to the U.S. Food and Drug Administration (the "FDA") in late 2019. In August 2020, we dosed the first patient with STK-001 in the single ascending dose portion of the MONARCH Phase 1/2a Study at the 10mg dose level.

In addition in November 2020, we announced the nomination of OPA1 as our next target for preclinical development to treat Autosomal Dominant Optic Atrophy ("ADOA"), and intend to invest significant efforts and financial resources in its development. 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 FDA or certain other foreign regulatory agencies, such as the European Medicines Agency (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 CTAs that allow commencement of our planned clinical trials or future clinical trials for our product candidates in relevant territories;
- potential delays in enrollment, site visits, evaluations, or dosing of patients participating in clinical trials as hospitals prioritize the treatment of COVID-19 patients or patients decide not to enroll in the study as a result of the COVID-19 pandemic;
- government regulations that may be imposed in response to the COVID-19 pandemic may restrict the movement of our global supply chain and divert hospital resources that are necessary to administer STK-001 or delay the review of future product candidates;
- the direct and indirect impact of COVID-19 on our business and operations, third party vendors, supply chain, and regulatory approvals;
- successful completion of preclinical studies, including those compliant with 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 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 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 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.

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 FDA or certain other foreign regulatory agencies, such as 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;
- potential delays in enrollment, site visits, evaluations, or dosing of patients participating in clinical trials as hospitals prioritize the treatment of COVID-19 patients or patients decide not to enroll in the study as a result of the COVID-19 pandemic;
- government regulations that may be imposed in response to the COVID-19 pandemic may restrict the movement of our global supply chain and divert hospital resources that are necessary to administer STK-001 or delay the review of future product candidates;
- the direct and indirect impact of COVID-19 on our business and operations, third party vendors, supply chain, and regulatory approvals;
- 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.

Success in early preclinical studies or clinical trials may not be indicative of results obtained in later preclinical studies and clinical trials, including in our Dravet syndrome program or our ADOA program.

STK-001 is currently being 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, and we may be similarly unable to demonstrate the efficacy of our future product candidates in our ADOA program or other future programs. Even if our clinical trials demonstrate acceptable safety and efficacy of STK-001 or our future product candidates, 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 for Dravet syndrome or a future product candidate for ADOA, 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. While currently we are not experiencing any significant delays or disruptions to our clinical trial a result of the global COVID-19 pandemic, we expect that the COVID-19 pandemic may directly or indirectly impact our clinical trial enrollment, dosing, and regulatory approval timelines.

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, any product candidate selected to treat ADOA, and our other future product candidates must be approved by the FDA pursuant to a new drug application (“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, any product candidate selected to treat ADOA or any of our other 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, any product candidate selected to treat ADOA and our other 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;
- 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;
- potential delays in enrollment, site visits, evaluations, or dosing of patients participating in the clinical trial as hospitals prioritize the treatment of COVID-19 patients or patients decide to not enroll in the study as a result of the COVID-19 pandemic; and
- government regulations that may be imposed in response to the COVID-19 pandemic may restrict the movement of our global supply chain, divert hospital resources that are necessary to administer STK-001 or our future product candidates.

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. While currently we are not experiencing any significant delays or disruptions to our clinical trial a result of the global COVID-19 pandemic, we expect that the COVID-19 pandemic may directly or indirectly impact our clinical trial enrollment, dosing, and regulatory approval timelines.

The ongoing COVID-19 pandemic may, directly or indirectly, adversely affect our business, results of operations and financial condition.

Our business could be materially adversely affected, directly or indirectly, by the widespread outbreak of contagious disease, including the ongoing COVID-19 pandemic, which has spread to many of the countries in which we and our suppliers do business. National, state and local governments in affected regions have implemented and may continue to implement safety precautions, including quarantines, border closures, increased border controls, travel restrictions, shelter in place orders and shutdowns, business closures, cancellations of public gatherings and other measures. Organizations and individuals are taking additional steps to avoid or

reduce infection, including limiting travel and staying home from work. These measures are disrupting normal business operations both in and outside of affected areas and have had significant negative impacts on businesses and financial markets worldwide.

The COVID-19 pandemic has caused us to modify our business practices (including but not limited to curtailing or modifying employee travel, moving to full remote work, and cancelling physical participation in meetings, events and conferences) and we may take further actions as may be required by government authorities or that we determine are in the best interests of our employees, patients and business partners. Our office-based employees have been working from home since early March 2020, while ensuring essential staffing levels in our operations remain in place, including maintaining key personnel in our laboratories.

Notwithstanding these measures, the COVID-19 pandemic could affect the health and availability of our workforce as well as those of the third parties we rely on taking similar measures. If members of our management and other key personnel in critical functions across our organization are unable to perform their duties or have limited availability due to COVID-19, we may not be able to execute on our business strategy and/or our operations may be negatively impacted. We may also experience limitations in employee resources, including because of sickness of employees or their families or the desire of employees to avoid contact with individuals or large groups of people. In addition, we have experienced and will continue to experience disruptions to our business operations resulting from quarantines, self-isolations and other restrictions on the ability of our employees to perform their jobs.

The COVID-19 pandemic has disrupted business operations. The extent and severity of the impact on our business and clinical trial will be determined largely by the extent of disruptions in the supply chains for STK-001 and our future product candidates in ADOA and other indications, and delays in the conduct of current and future clinical trials. Our ability to continue our observational study may be adversely affected, directly or indirectly, by the COVID-19 pandemic. Currently we are monitoring patient enrollment in our observational study, including the duration and degree to which we may see declines in enrollment, delays in conducting in-person follow-ups, and disruptions in our ability to monitor patients due to hospitals closing sites or diverting the resources that are necessary to conduct our observational study to care for COVID-19 patients. We expect that COVID-19 precautions may directly or indirectly impact the timeline for some of our clinical trial activities due to hospitals closing sites and/or diverting the resources that are necessary to conduct our observational study to care for COVID-19 patients. In addition, the impact of the COVID-19 pandemic on the operations of the FDA and other health authorities may delay potential approvals of STK-001 and our future product candidates.

While it is not possible at this time to estimate the entirety of the impact that the COVID-19 pandemic will have on our business, operations, employees, customers, or our suppliers, continued spread of COVID-19, measures taken by governments, actions taken to protect employees and the broad impact of the pandemic on all business activities may materially and adversely affect our business, results of operations and financial condition.

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 worldwide incidence of Dravet syndrome is approximately one in 16,000 births, and the incidence of ADOA is approximately one in 30,000 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.

Any 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 or ADOA, 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 and ADOA, our per-patient therapy pricing of STK-001 and our future product candidates, 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. While currently we are not experiencing any significant delays or disruptions to our clinical trial as a result of the global COVID-19 pandemic, we expect that the COVID-19 pandemic may directly or indirectly impact our clinical trial enrollment, dosing, and regulatory approval timelines.

If clinical trials of STK-001, the product candidate we select to treat ADOA or any other product candidate that we develop fail to demonstrate safety and efficacy to the satisfaction of FDA or foreign regulatory authorities or do not otherwise produce favorable results, we may incur additional costs or experience delays in completing, or ultimately may be unable to complete, the development and commercialization of such product candidate.

Before obtaining marketing approval from regulatory authorities for the sale of any product candidate, including STK-001 and the product candidate we select to treat ADOA, we must complete preclinical development and then conduct extensive clinical trials to demonstrate the safety and efficacy of our product candidates in humans. Clinical testing is expensive, difficult to design and implement, can take many years to complete and is uncertain as to outcome. A failure of one or more clinical trials can occur at any stage of testing.

Clinical trials may be placed on a full or partial clinical hold by the FDA, foreign regulatory authorities, or us for various reasons, including but not limited to: deficiencies in the conduct of the clinical trials, including failure to conduct the clinical trial in accordance with regulatory requirements or clinical protocols; deficiencies in the clinical trial operations or trial sites; deficiencies in the trial designs necessary to demonstrate efficacy; fatalities or other adverse effects arising during a clinical trial due to medical problems that may or may not be related to clinical trial treatments; the product candidates may not appear to be more effective than current therapies; the quality or stability of the product candidates may fall below acceptable standards; or the data from animal studies are not sufficient to support the anticipated exposure (dose, route of administration, and duration) for the proposed clinical trial. For example, in March 2020, we announced that the FDA had placed a partial clinical hold on doses of STK-001 above 20 mg in our Phase 1/2a clinical trial (the "MONARCH Study") based on observations of adverse hind limb paresis in non-human primates, pending additional preclinical testing to determine the safety profile of doses higher than the current no observed adverse effect level. The partial clinical hold remains in place in the MONARCH Study for dosing above 30 mg. If the partial clinical hold is not lifted, our ability to successfully conclude the MONARCH Study or other studies related to STK-001, and our business, results of operations and financial condition, may be adversely affected.

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.

In November 2020, we announced the nomination of OPA1 as our second program for preclinical development in ADOA; however, 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 ("GMPs"), 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, the product candidate we select to treat ADOA 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 (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 or our future product candidates, including the product candidate we select to treat ADOA, 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 or our future product candidates, including the product candidate we select to treat ADOA, and drugs in the antisense oligonucleotide class.

In addition to side effects caused by our product candidates, the intrathecal or intravitreal 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 in the labeling;
- 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 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.

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 for any of our product candidates, 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. Full approval of another product for the same indication as any of our product candidates for which we are seeking accelerated approval may make accelerated approval of our product candidates more difficult. 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 general the FDA may require that the trial be designed and/or initiated prior to approval. All promotional materials for product candidates approved via accelerated approval are subject to prior review by the FDA. 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, including the product candidate we select to treat ADOA, 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, the product candidate we select to treat ADOA 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.

In the United States, there have been and continue to be a number of legislative initiatives to contain healthcare costs. 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 Patient Protection and Affordable Care Act, as amended by the Health Care and Education Reconciliation Act of 2010 (collectively, the "ACA") was enacted, which was 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 health care and health insurance industries, impose new taxes and fees on the health industry and impose additional health policy reforms. The ACA substantially changed the way healthcare is financed by both governmental and private insurers, and significantly impacts the U.S. pharmaceutical industry. Various portions of the ACA are currently undergoing legal and constitutional challenges in the United States Supreme Court; the Trump Administration issued various Executive Orders that eliminated cost sharing subsidies and various provisions that would impose a fiscal burden on states or a cost, fee, tax, penalty or regulatory burden on individuals, healthcare providers, health insurers, or manufacturers of pharmaceuticals or medical devices; and Congress has introduced several pieces of legislation aimed at significantly revising or repealing the ACA. Although the current U.S. presidential administration has sought to strengthen the ACA, it remains unclear whether the ACA will be overturned, repealed, replaced, or further amended. We cannot predict what affect further changes to the ACA would have on our business.

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 2030 unless additional Congressional action is taken. The CARES Act, which was signed into law in March 2020 and is designed to provide financial support and resources to individuals and businesses affected by the COVID-19 pandemic, suspended the 2% Medicare sequester from May 1, 2020 through December 31, 2020. 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 1 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, the product candidate we select to treat ADOA or our future product candidates.

As part of our business strategy, we applied for and, in August 2019, received 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, and there is no guarantee that we will be successful in obtaining such designation for 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.

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 our future product candidates, including the product candidate we select to treat ADOA, 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 a NDA. We may seek Rare Pediatric Disease designations for STK-001 or any future product candidates. If a product candidate is designated before December 11, 2020, it is eligible to receive a voucher if it is approved before December 11, 2022. However, there is no expectation that STK-001 or our future product candidates, including the product candidate we select to treat ADOA, will be designated or approved by those dates, 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. The Creating Hope Authorization Act (H.R. 4439), which has been passed by the House of Representatives, but not the Senate, would further extend the designation deadline to September 30, 2024, and the approval deadline to September 30, 2026. There is no guarantee that this bill will become law or that the deadlines will be extended. 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, marketing expenditures or pricing. 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 and the product candidate we select to treat ADOA 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, the product we select to treat ADOA and any future product candidates, including acceptance of intravitreal injection, 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 and ADOA, 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 (“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.

The CARES Act, and other potential future healthcare reforms may adversely impact pricing, insurance coverage and reimbursement status of newly approved products.

In response to the COVID-19 pandemic, the Coronavirus Aid, Relief and Economic Security Act (the “CARES Act") was signed into law in March 2020. The CARES Act is aimed at providing emergency assistance and health care for individuals, families and businesses affected by the COVID-19 pandemic and generally supporting the U.S. economy. Due to the recent enactment of the CARES Act, there is a high degree of uncertainty around its implementation. We expect that additional state and federal healthcare reform measures may be adopted in the future, any of which could limit the amounts that federal and state governments will pay for healthcare products and services, which could result in reduced demand for our future products or additional pricing pressures.

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, the product candidate we select to treat ADOA 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.

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, the product candidate we select to treat ADOA 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, the product candidate we select to treat ADOA 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, the product candidate we select to treat ADOA 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 net losses of $16.8 million and $11.0 million, for the quarter ended March 31, 2021 and 2020, respectively. As of March 31, 2021 and 2020, we had accumulated deficits of $127.1 million and $69.1 million, respectively. Substantially all 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, the product candidate we select to treat ADOA 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, the product candidate we select to treat ADOA and other future product candidates, if any, and potentially commercialize these product candidates. Based upon our current operating plan, we believe that our cash, cash equivalents and restricted cash of $267.7 million as of March 31, 2021, will enable us to fund our operating expenses and capital expenditure requirements into 2024. 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 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 a clinical 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, preclinical and clinical development 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, 2020, we had federal and state net operating loss carryforwards, or NOLs, of approximately $116.9 million and $118.8 million, respectively, and as of December 31, 2019, we had federal and state NOLs of approximately $54.5 million and $56.3 million, respectively. Our NOLs 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. 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, the CARES Act temporarily repealed the 80% taxable income limitation for tax years beginning before January 1, 2021. Federal NOL carry forwards generated from 2018 or later and Federal NOL carryforwards to taxable years beginning after December 31, 2020 will be subject to the 80% limitation. Also, under the CARES Act, federal NOLs arising in 2018, 2019 and 2020 can be carried back 5 years. 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 own an issued U.S. patent covering STK-001 and related compositions, an issued U.S. patent covering the mechanism of action of STK-001 and use of STK-001 for treating diseases, and our U.S. patent application covering the mechanism of action of STK-001 and use of STK-001 for treating diseases is currently pending. We have also in-licensed two issued U.S. patents that covers the mechanism of action of STK-001, use of the mechanism for treating diseases, and related compositions. We have obtained at least two issued foreign patents covering STK-001 and related compositions and are currently pursuing patent protection for STK-001, related compositions, and its uses in several economically significant countries. With respect to our ADOA program (modulating OPA1 gene output), we have applied for and are currently pursuing patent protection for the mechanism of action and methods of treatment in several economically significant countries. We have also applied for a U.S. patent application that specifically discloses compositions related to our ADOA program and uses of those compositions. Furthermore, our in-licensed issued U.S. patents (mentioned above) cover the mechanism of action of modulating OPA1 gene output. 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 and other programs 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 (“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 (the "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 2042, 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 (the "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, the 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 (the "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 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 (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, our Chief Financial Officer, our Chief Operating Officer and Chief Business Officer, our Chief Medical Officer, our Chief Scientific Officer, and our Co-Founder and Group VP, Discovery Research, 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.

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.

If members of our management and other key personnel in critical functions across our organization are unable to perform their duties or have limited availability due to the COVID-19 pandemic, we may not be able to execute on our business strategy and/or our operations may be negatively impacted. Our success is dependent upon our ability to attract and retain qualified management and key personnel in a highly competitive environment. Qualified individuals are in high demand, and we may incur significant costs to attract them, particularly at the executive level. We may face difficulty in attracting and retaining key talent for a number of reasons, including management changes, the underperformance or discontinuation of our clinical stage program, recruitment by competitors or delays in the recruiting and hiring process as a result of the COVID-19 pandemic. We cannot ensure that we will be able to hire or retain the personnel necessary for our operations or that the loss of any such personnel will not have a material impact on our financial condition and results of operations.

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. As another example, our financial results may be negatively impacted by the recent COVID-19 outbreak. The extent and duration of such impacts remain largely uncertain and dependent on future developments that cannot be accurately predicted at this time, such as the severity and transmission rate of COVID-19, the extent and effectiveness of containment actions taken and the impact of these and other factors on our operations and the global economy in general. 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 our third party service providers, face risks related to health epidemics and other outbreaks, which could significantly disrupt our operations.

Our business could be adversely impacted by the effects of COVID-19 or other epidemics. A public health epidemic, including COVID-19, poses the risk that we or our employees, contractors, suppliers, and other partners may be prevented from conducting business activities for an indefinite period of time, including due to shutdowns that may be requested or mandated by governmental authorities. We currently rely, and may continue to rely, on third-party service providers that are located in locals significantly impacted by COVID-19 and/or who source raw materials, samples, components, or other materials and reports from countries significantly impacted by COVID-19. Consequently, supply of research materials and early research activities are susceptible to factors adversely affecting one or more of our third-party service providers who are located in and/or who source from locations significantly impacted by COVID-19. We may also experience impacts to certain of our suppliers as a result of COVID-19 or other health epidemic or outbreak occurring in one or more of these locations, which may materially and adversely affect our business, financial condition and results of operations. In addition, hospitals or other clinical trial sites could also become overwhelmed by COVID-19 and shift resources or attention away from our clinical trials, which may cause delays in our trials or negatively affect enrollment. COVID-19 and mitigation measures may also have an adverse impact on global economic conditions which could have an adverse effect on our business and financial condition. The extent to which COVID-19 impacts our results will depend on future developments that are highly uncertain and cannot be predicted, including new information that may emerge concerning the severity of the virus and the actions to contain its impact.

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.

A breakdown or breach of our technology systems could subject us to liability or interrupt the operation of our business.

We are increasingly dependent upon technology systems and data to operate our business. In particular, the COVID-19 pandemic has caused us to modify our business practices, including the requirement that our office-based employees in the U.S. work from home. As a result, we are increasingly dependent upon our technology systems to operate our business and our ability to effectively manage our business depends on the security, reliability and adequacy of our technology systems and data, which includes use of cloud technologies, including Software as a Service (SaaS), Platform as a Service (PaaS) and Infrastructure as a Service (IaaS). A breakdown, invasion, corruption, destruction or breach of our technology systems, including the cloud technologies that we utilize, and/or unauthorized access to our data and information could subject us to liability or negatively impact the operation of our business. Our technology systems, including the cloud technologies that we utilize, continue to increase in multitude and complexity, making them potentially vulnerable to breakdown, malicious intrusion and random attack. Likewise, data privacy or security breaches by individuals authorized to access our technology systems, including the cloud technologies that we utilize, may pose a risk that sensitive data, including intellectual property, trade secrets or personal information belonging to us, our patients or other business partners, may be exposed to unauthorized persons or to the public.

Cyberattacks are increasing in their frequency, sophistication and intensity, and have become increasingly difficult to detect. They are often carried out by motivated, well-resourced, skilled and persistent actors, including nation states, organized crime groups, “hacktivists” and employees or contractors acting with malicious intent. Cyber-attacks could include the deployment of harmful malware and key loggers, ransomware, a denial-of-service attack, a malicious website, the use of social engineering and other means to affect the confidentiality, integrity and availability of our technology systems and data. Cyber-attacks could also include supply chain attacks, which could cause a delay in the manufacturing of our products or products produced for contract manufacturing. Our key business partners face similar risks and any security breach of their systems could adversely affect our security posture. 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.

Moreover, our increased use of cloud technologies could heighten these and other operational risks, and any failure by cloud technology service providers to adequately safeguard their systems and prevent cyber-attacks could disrupt our operations and result in misappropriation, corruption or loss of confidential or propriety information. 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. While we continue to build and improve our systems and infrastructure, including our business continuity plans, there can be no assurance that our efforts will prevent breakdowns or breaches in our systems that could adversely affect our business and operations and/or result in the loss of critical or sensitive information, which could result in financial, legal, business, operational or reputational harm to us, or loss of competitive advantage. In addition, our liability insurance may not be sufficient in type or amount to cover us against claims related to security breaches, cyber-attacks and other related 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 ownership of our common stock

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 may be highly volatile and subject to wide fluctuations in response to various factors, some of which we cannot control. The market price for our common stock may be influenced by many factors, including the other risks described in this section and elsewhere in this report 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;
- the impact of the COVID-19 pandemic on our employees, trials, collaboration partners, suppliers, our results of operations, liquidity and financial condition;
- 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.

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.

Our executive officers, directors and affiliates control a significant portion of our outstanding voting stock. As a result, these stockholders, if acting together, will likely continue to have effective control 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.

In addition, as of November 20, 2020, an entity affiliated with Apple Tree Partners beneficially owns 47.5% of the voting power of all outstanding shares of our common stock. This entity previously controlled a majority of the voting power of our common stock and, accordingly, we previously qualified as a "controlled company" within the meaning of the Nasdaq listing rules. As such, we were permitted to rely upon certain scaled governance requirements, although we elected not to do so.

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 have any control over the analysts, or the content and opinions included in their reports. 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.

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 (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 (the "Sarbanes-Oxley Act"), (ii) reduced disclosure obligations regarding executive compensation in 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.

We will remain an emerging growth company until the earlier of (i) the last day of the fiscal year (a) in which we have total annual gross revenue of at least $1.07 billion or (b) in which we are deemed to be a large accelerated filer, which requires the market value of our common stock that is held by non-affiliates to exceed $700 million as of the prior June 30th, (ii) the date on which we have issued more than $1.0 billion in non-convertible debt during the prior three-year period and (iii) December 31, 2024.

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 was less than $700 million and our annual revenue was less than $100 million during the most recently completed fiscal year. We may continue to be a

smaller reporting company as long as 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. 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.

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 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.

The exclusive forum provision in our restated certificate of incorporation 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.

Our restated certificate of incorporation, to the fullest extent permitted by law, provides that the Court of Chancery of the State of Delaware is 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 (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.

Section 22 of the Securities Act creates concurrent jurisdiction for federal and state courts over all claims brought to enforce any duty or liability created by the Securities Act or the rules and regulations thereunder. In April 2020, we amended and restated our restated bylaws to provide that the federal district courts of the United States will, to the fullest extent permitted by law, be the exclusive forum for resolving any complaint asserting a cause of action arising under the Securities Act (such provision, a "Federal Forum Provision"). Our decision to adopt a Federal Forum Provision followed a decision by the Supreme Court of the State of Delaware holding that such provisions are facially valid under Delaware law. While there can be no assurance that federal or state courts will follow the holding of the Delaware Supreme Court or determine that the Federal Forum Provision should be enforced in a particular case, application of the Federal Forum Provision means that suits brought by our stockholders to enforce any duty or liability created by the Securities Act must be brought in federal court and cannot be brought in state court.

Section 27 of the Exchange Act creates exclusive federal jurisdiction over all claims brought to enforce any duty or liability created by the Exchange Act or the rules and regulations thereunder. In addition, neither the exclusive forum provision nor the Federal Forum Provision applies to suits brought to enforce any duty or liability created by the Exchange Act. Accordingly, actions by our stockholders to enforce any duty or liability created by the Exchange Act or the rules and regulations thereunder must be brought in federal court.

Our stockholders will not be deemed to have waived our compliance with the federal securities laws and the regulations promulgated thereunder.

Any person or entity purchasing or otherwise acquiring or holding any interest in any of our securities shall be deemed to have notice of and consented to our exclusive forum provisions, including the Federal Forum Provision. These provisions may limit a stockholder's ability to bring a claim in a judicial forum of their choosing for disputes with us or our directors, officers, or other employees, which may discourage lawsuits against us and our directors, officers, and other employees.

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 Select 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. 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 previously were not required to independently comply with Section 404(a) of the Sarbanes-Oxley Act. Section 404(a) of the Sarbanes-Oxley Act requires annual management assessments of the effectiveness of our internal control over financial reporting, starting with the second annual report that we file with the SEC. We were required to meet these standards in the course of preparing our financial statements as of and for the year ended December 31, 2020, and our management is required to report on the effectiveness of our internal control over financial reporting for such year and annually thereafter. Additionally, once we are no longer an "emerging growth company," our independent registered public accounting firm will be required pursuant to Section 404(b) of the Sarbanes-Oxley Act to attest to the effectiveness of our internal control over financial reporting on an annual basis. The rules governing the standards that must be met for our management to assess our internal control over financial reporting are complex and require significant documentation, testing, and possible remediation.

To achieve compliance with Section 404(b) 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.

Item 2. Unregistered Sales of Equity Securities and Use of Proceeds.

Unregistered Sales of Equity Securities

None.

Use of Proceeds

On November 24, 2020, we completed an underwritten public offering (the "Follow-On Offering"), of our common stock and issued and sold 2,875,000 shares of common stock at a public offering price of $39.00 per share, which included 375,000 shares sold upon full exercise of the underwriters' option to purchase additional shares of common stock resulting in net proceeds of $104.9 million after deducting underwriting discounts and commissions and estimated offering expenses. J.P. Morgan Securities LLC, Cowen and Company, LLC and Credit Suisse Securities (USA) LLC acted as joint book-running managers of the offering and as representatives of the underwriters. None of the expenses associated with the Follow-On Offering were paid to directors, officers, persons owning 10% or more of any class of equity securities, or to their associates, or to our affiliates.

On June 21, 2019, we completed our IPO and sold 9,074,776 shares of common stock at an IPO price of $18.00 per share. The offer and sale of all of the shares in the IPO were registered under the Securities Act pursuant to registration statements on Form S-1 (File Nos. 333-231700 and 333-232192), which was declared effective by the SEC on June 18, 2019. No additional shares were registered.

We received net proceeds from the IPO of approximately $149.4 million, after deducting underwriting discounts and commissions of approximately $11.4 million and estimated offering expenses of approximately $2.5 million. J.P. Morgan Securities LLC, Cowen and Company, LLC and Credit Suisse Securities (USA) LLC acted as joint book-running managers of the offering and as representatives of the underwriters. None of the expenses associated with the IPO were paid to directors, officers, persons owning 10% or more of any class of equity securities, or to their associates, or to our affiliates.

There has been no material change in the planned use of proceeds from our IPO as described in the Prospectus filed with the SEC pursuant to Rule 424(b)(4) under the Securities Act on June 19, 2019.

Item 3. Defaults Upon Senior Securities.

None.

Item 4. Mine Safety Disclosures.

Not Applicable.

Item 5. Other Information.

None.

Item 6. Exhibits.

The exhibits filed or furnished as part of this Quarterly Report on Form 10-Q are set forth on the Exhibit Index below.

Exhibit Number	Description	Form	File No.	Exhibit Filing Date	Exhibit No.	Filed/Furnished Herewith
31.1	Certification of Principal Executive Officer Pursuant to Rules 13a-14(a) and 15d-14(a) under the Securities Exchange Act of 1934, as Adopted Pursuant to Section 302 of the Sarbanes-Oxley Act of 2002.					X
31.2	Certification of Principal Financial Officer Pursuant to Rules 13a-14(a) and 15d-14(a) under the Securities Exchange Act of 1934, as Adopted Pursuant to Section 302 of the Sarbanes-Oxley Act of 2002.					X
32.1*	Certification of Principal Executive Officer Pursuant to 18 U.S.C. Section 1350, as Adopted Pursuant to Section 906 of the Sarbanes-Oxley Act of 2002.					X
32.2*	Certification of Principal Financial Officer Pursuant to 18 U.S.C. Section 1350, as Adopted Pursuant to Section 906 of the Sarbanes-Oxley Act of 2002.					X
101.INS	XBRL Instance Document.					X
101.SCH	XBRL Taxonomy Extension Schema Document.					X
101.CAL	XBRL Taxonomy Extension Calculation Linkbase Document.					X
101.DEF	XBRL Taxonomy Extension Definition Linkbase Document.					X
101.LAB	XBRL Taxonomy Extension Label Linkbase Document.					X
101.PRE	XBRL Taxonomy Extension Presentation Linkbase Document.					X

* This certification is deemed not filed for purposes of section 18 of the Exchange Act or otherwise subject to the liability of that section, nor shall it be deemed incorporated by reference into any filing under the Securities Act of the Exchange Act.

SIGNATURES

Pursuant to the requirements of the Securities Exchange Act of 1934, the registrant has duly caused this report to be signed on its behalf by the undersigned thereunto duly authorized.

STOKE THERAPEUTICS, INC.

Date: May 10, 2021 By: /s/ Edward M. Kaye, M.D.

Edward M. Kaye, M.D.
Chief Executive Officer
(Principal Executive Officer)

Date: May 10, 2021 By: /s/ Stephen J. Tulipano

Stephen J. Tulipano
Chief Financial Officer
(Principal Financial Officer and Principal Accounting Officer)

Exhibit 31.1

CERTIFICATION PURSUANT TO RULE 13a-14(a) OR 15d-14(a) OF THE SECURITIES EXCHANGE ACT OF 1934, AS ADOPTED PURSUANT TO SECTION 302 OF THE SARBANES-OXLEY ACT OF 2002

I, Edward M. Kaye, certify that:

1. I have reviewed this Quarterly Report on Form 10-Q of Stoke Therapeutics, Inc.;

2. Based on my knowledge, this report does not contain any untrue statement of a material fact or omit to state a material fact necessary to make the statements made, in light of the circumstances under which such statements were made, not misleading with respect to the period covered by this report;

3. Based on my knowledge, the financial statements, and other financial information included in this report, fairly present in all material respects the financial condition, results of operations and cash flows of the registrant as of, and for, the periods presented in this report;

4. The registrant's other certifying officer and I are responsible for establishing and maintaining disclosure controls and procedures (as defined in Exchange Act Rules 13a-15(e) and 15d-15(e)) for the registrant and have:

   a. Designed such disclosure controls and procedures, or caused such disclosure controls and procedures to be designed under our supervision, to ensure that material information relating to the registrant, including its consolidated subsidiaries, is made known to us by others within those entities, particularly during the period in which this report is being prepared;

   b. Designed such internal control over financial reporting, or caused such internal control over financial reporting to be designed under our supervision, to provide reasonable assurance regarding the reliability of financial reporting and the preparation of financial statements for external purposes in accordance with generally accepted accounting principles;

   c. Evaluated the effectiveness of the registrant's disclosure controls and procedures and presented in this report our conclusions about the effectiveness of the disclosure controls and procedures, as of the end of the period covered by this report based on such evaluation; and

   d. Disclosed in this report any change in the registrant's internal control over financial reporting that occurred during the registrant's most recent fiscal quarter (the registrant's fourth fiscal quarter in the case of an annual report) that has materially affected, or is reasonably likely to materially affect, the registrant's internal control over financial reporting; and

5. The registrant's other certifying officer and I have disclosed, based on our most recent evaluation of internal control over financial reporting, to the registrant's auditors and the audit committee of the registrant's board of directors (or persons performing the equivalent functions):

   a. All significant deficiencies and material weaknesses in the design or operation of internal control over financial reporting which are reasonably likely to adversely affect the registrant's ability to record, process, summarize and report financial information; and
   b. Any fraud, whether or not material, that involves management or other employees who have a significant role in the registrant's internal control over financial reporting.

Date: May 10, 2021

/s/ Edward M. Kaye, M.D.
Edward M. Kaye, M.D.
Chief Executive Officer
(Principal Executive Officer)

Exhibit 31.2

CERTIFICATION PURSUANT TO RULE 13a-14(a) OR 15d-14(a) OF THE SECURITIES EXCHANGE ACT OF 1934, AS ADOPTED PURSUANT TO SECTION 302 OF THE SARBANES-OXLEY ACT OF 2002

I, Stephen J. Tulipano, certify that:

1. I have reviewed this Quarterly Report on Form 10-Q of Stoke Therapeutics, Inc.;

2. Based on my knowledge, this report does not contain any untrue statement of a material fact or omit to state a material fact necessary to make the statements made, in light of the circumstances under which such statements were made, not misleading with respect to the period covered by this report;

3. Based on my knowledge, the financial statements, and other financial information included in this report, fairly present in all material respects the financial condition, results of operations and cash flows of the registrant as of, and for, the periods presented in this report;

4. The registrant's other certifying officer and I are responsible for establishing and maintaining disclosure controls and procedures (as defined in Exchange Act Rules 13a-15(e) and 15d-15(e)) for the registrant and have:

   a. Designed such disclosure controls and procedures, or caused such disclosure controls and procedures to be designed under our supervision, to ensure that material information relating to the registrant, including its consolidated subsidiaries, is made known to us by others within those entities, particularly during the period in which this report is being prepared;

   b. Designed such internal control over financial reporting, or caused such internal control over financial reporting to be designed under our supervision, to provide reasonable assurance regarding the reliability of financial reporting and the preparation of financial statements for external purposes in accordance with generally accepted accounting principles;

   c. Evaluated the effectiveness of the registrant's disclosure controls and procedures and presented in this report our conclusions about the effectiveness of the disclosure controls and procedures, as of the end of the period covered by this report based on such evaluation; and

   d. Disclosed in this report any change in the registrant's internal control over financial reporting that occurred during the registrant's most recent fiscal quarter (the registrant's fourth fiscal quarter in the case of an annual report) that has materially affected, or is reasonably likely to materially affect, the registrant's internal control over financial reporting; and

5. The registrant's other certifying officer and I have disclosed, based on our most recent evaluation of internal control over financial reporting, to the registrant's auditors and the audit committee of the registrant's board of directors (or persons performing the equivalent functions):

   a. All significant deficiencies and material weaknesses in the design or operation of internal control over financial reporting which are reasonably likely to adversely affect the registrant's ability to record, process, summarize and report financial information; and
   b. Any fraud, whether or not material, that involves management or other employees who have a significant role in the registrant's internal control over financial reporting.

Date: May 10, 2021

/s/ Stephen J. Tulipano, CPA
Stephen J. Tulipano, CPA
Chief Financial Officer
(Principal Financial Officer and Principal Accounting Officer)

Exhibit 32.1

CERTIFICATION PURSUANT TO
18 U.S.C. SECTION 1350, AS ADOPTED PURSUANT TO
SECTION 906 OF THE SARBANES-OXLEY ACT OF 2002

I, Edward M. Kaye, Chief Executive Officer of Stoke Therapeutics, Inc. (the "Company"), do hereby certify, pursuant to 18 U.S.C. Section 1350, as adopted pursuant to Section 906 of the Sarbanes-Oxley Act of 2002, that to the best of my knowledge:

1. the Quarterly Report on Form 10-Q of the Company for the fiscal quarter ended March 31, 2021 (the "Report") fully complies with the requirements of Section 13(a) or 15(d) of the Securities Exchange Act of 1934, as amended; and

2. the information contained in the Report fairly presents, in all material respects, the financial condition and results of operations of the Company.

Dated: May 10, 2021

/s/ Edward M. Kaye, M.D.
Edward M. Kaye, M.D.
Chief Executive Officer
(Principal Executive Officer)

Exhibit 32.2

CERTIFICATION PURSUANT TO
18 U.S.C. SECTION 1350, AS ADOPTED PURSUANT TO
SECTION 906 OF THE SARBANES-OXLEY ACT OF 2002

I, Stephen J. Tulipano, Chief Financial Officer of Stoke Therapeutics, Inc. (the "Company"), do hereby certify, pursuant to 18 U.S.C. Section 1350, as adopted pursuant to Section 906 of the Sarbanes-Oxley Act of 2002, that to the best of my knowledge:

1. the Quarterly Report on Form 10-Q of the Company for the fiscal quarter ended March 31, 2021 (the "Report") fully complies with the requirements of Section 13(a) or 15(d) of the Securities Exchange Act of 1934, as amended; and

2. the information contained in the Report fairly presents, in all material respects, the financial condition and results of operations of the Company.

Dated: May 10, 2021

/s/ Stephen J. Tulipano, CPA
Stephen J. Tulipano, CPA
Chief Financial Officer
(Principal Financial Officer and
Principal Accounting Officer)