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

FORM 8-K

CURRENT REPORT
Pursuant to Section 13 or 15(d)
of the Securities Exchange Act of 1934

Date of Report (Date of earliest event reported): January 9, 2022

Stoke Therapeutics, Inc.
(Exact Name of Registrant as Specified in its Charter)

Delaware	**001-38938**	**47-1144582**
(State or other jurisdiction of incorporation or organization)	**(Commission File Number)**	**(I.R.S. Employer Identification No.)**

45 Wiggins Ave **Bedford, Massachusetts**	**01730**
(Address of principal executive offices)	**(Zip Code)**

Registrant's telephone number, including area code: (781) 430-8200

Not Applicable
(Former Name or Former Address, if Changed Since Last Report)

Check the appropriate box below if the Form 8-K filing is intended to simultaneously satisfy the filing obligation of the registrant under any of the following provisions:

☐ Written communications pursuant to Rule 425 under the Securities Act (17 CFR 230.425)

☐ Soliciting material pursuant to Rule 14a-12 under the Exchange Act (17 CFR 240.14a-12)

☐ Pre-commencement communications pursuant to Rule 14d-2(b) under the Exchange Act (17 CFR 240.14d-2(b))

☐ Pre-commencement communications pursuant to Rule 13e-4(c) under the Exchange Act (17 CFR 240.13e-4(c))

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 is an emerging growth company as defined in Rule 405 of the Securities Act of 1933 (§230.405 of this chapter) or Rule 12b-2 of the Securities Exchange Act of 1934 (§240.12b-2 of this chapter).

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. ☐

Item 1.01. Entry into a Material Definitive Agreement.

On January 9, 2022, Stoke Therapeutics, Inc., a Delaware corporation (the "Company") entered into a License and Collaboration Agreement (the "Agreement") with Acadia Pharmaceuticals Inc. ("Acadia") for the discovery, development and commercialization of novel RNA-based medicines for the treatment of severe and rare genetic neurodevelopmental disease of the central nervous system ("CNS"). The Agreement focuses on the targets SYNGAP1, MECP2 (Rett syndrome), and an undisclosed neurodevelopmental target of mutual interest. In connection with each target, the parties will collaborate to identify potential treatments for further development and commercialization as licensed products. With respect to SYNGAP1, the parties have agreed to co-develop and co-commercialize licensed products for such target globally, and in connection therewith the Company granted to Acadia worldwide, co-exclusive (with the Company) licenses for such licensed products. With respect to MECP2 and the neurodevelopmental target, the Company granted to Acadia worldwide, exclusive licenses to develop and commercialize licensed products for such targets.

Pursuant to the Agreement, Acadia will pay the Company an upfront payment in the amount of $60,000,000. Acadia will fund the research to identify potential licensed products for MECP2 and the neurodevelopmental target, and the parties will equally fund the research to identify potential licensed products for SYNGAP1. The Company is eligible to receive up to $907,500,000 in potential total milestone payments based upon the achievement of certain development, regulatory, first commercial sales and sales milestone events across the programs for the three targets, assuming each milestone were achieved at least once. With respect to licensed products for MECP2 and the neurodevelopmental target, the Company is also eligible to receive tiered royalties at percentages ranging from the mid-single digits to the mid-teens on future net sales by Acadia of licensed products worldwide. Royalties payable under the Agreement are subject to standard royalty reductions. For SYNGAP1 licensed products that the parties are co-developing and co-commercializing, the Company will be responsible for 50% of the development and commercialization costs and will receive 50% of the profits from global commercialization.

With respect to each SYNGAP1 licensed product being co-developed or co-commercialized, the Agreement will remain in effect, unless earlier terminated, until the parties have agreed to permanently abandon the further development and commercialization of such licensed product. With respect to licensed products for MECP2 and the neurodevelopmental target, the Agreement will remain in effect, unless earlier terminated, until the expiration, on a country-by-country and licensed product-by-licensed product basis, of the applicable royalty term, at which point the license for such licensed product shall become fully paid-up, royalty-free, perpetual and irrevocable in such country.

The Agreement also contains customary provisions for termination by Acadia for convenience and by either party for cause, including for material breach (subject to cure). The Company has standard reversion rights in connection with certain early termination events.

The foregoing description of the terms of the Agreement does not purport to be complete and is qualified in its entirety by reference to the full text of the Agreement, a copy of which will be filed with the Securities and Exchange Commission as an exhibit to the Company's Quarterly Report on Form 10-Q for the quarter ending March 30, 2022.

Item 2.02. Results of Operations and Financial Condition

On January 10, 2022, the Company plans to present certain preliminary financial and operating information in connection with a presentation (the "Presentation") at the J.P. Morgan Healthcare Conference, including that the Company expects to report that it had cash, cash equivalents, marketable securities and restricted cash of approximately $220.4 million, and approximately 36.9 million shares of common stock outstanding, as of December 31, 2021.

The Company's audited financial statements for the fiscal year ended December 31, 2021, are not yet available. Accordingly, the preliminary financial information included in the Presentation is an estimate subject to the completion of the Company's financial closing procedures and any adjustments that may result from the completion of the audit of the Company's financial statements. The preliminary financial information may differ materially from the actual results that will be reflected in the Company's audited financial statements when they are completed and publicly disclosed.

The information in this Item 2.02 shall not be deemed "filed" for purposes of Section 18 of the Securities Exchange Act of 1924, as amended (the "Exchange Act"), or otherwise subject to the liabilities of that section, nor shall it be deemed incorporated by reference into any other filing under the Exchange Act or the Securities Act of 1933, as amended (the "Securities Act"), except as expressly set forth by specific reference in such a filing.

Item 7.01. Regulation FD.

Presentation

The Company is furnishing the Presentation, a full copy of which is attached hereto as Exhibit 99.1.

Press Release

On January 10, 2022, the Company and Acadia issued a joint press release, announcing their entry into the Agreement. A copy of the press release is attached hereto as Exhibit 99.2.

The information furnished with this report, including Exhibit 99.1 and Exhibit 99.2, shall not be deemed "filed" for purposes of Section 18 of the Exchange Act, or otherwise subject to the liabilities of that section, nor shall it be deemed incorporated by reference into any other filing under the Exchange Act or the Securities Act, except as expressly set forth by specific reference in such a filing.

Item 8.01 Other Events

The Company also reported that its cash, cash equivalents, marketable securities and restricted cash, including the upfront payment payable by Acadia pursuant to the Agreement, are expected to fund operations into the second half of 2024.

Cautionary Note Regarding Forward-Looking Statements

This report contains forward-looking statements within the meaning of the "safe harbor" provisions of the Private Securities Litigation Reform Act of 1995, including, but not limited to, statements regarding: receipt of upfront payments; receipt of potential milestone payments under the SYNGAP1 collaboration; receipt of potential milestones and royalty payments under the MECP2 program and the third program; the ability to develop new treatments for neurodevelopmental diseases; expectations regarding the proposed transaction with Acadia; and the Company's expected cash, cash equivalents, marketable securities and restricted cash as of December 31, 2021. Statements including words such as "believe," "plan," "will," "continue," "expect," "may," or "ongoing" and statements in the future tense are forward-looking statements. These forward-looking statements involve risks and uncertainties, as well as assumptions, which, if they do not fully materialize or prove incorrect, could cause the Company's results to differ materially from those expressed or implied by such forward-looking statements. Forward-looking statements are subject to risks and uncertainties that may cause the company's actual activities or results to differ significantly from those expressed in any forward-looking statement, including risks and uncertainties related to the Company's ability to advance its product candidates, obtain regulatory approval of and ultimately commercialize its product candidates, the timing and results of preclinical and clinical trials, the company's ability to fund development activities and achieve development goals, the Company's ability to protect intellectual property and other risks and uncertainties described under the heading "Risk Factors" in documents the Company files from time to time with the Securities and Exchange Commission. These forward-looking statements speak only as of the date of this press release, and the Company undertakes no obligation to revise or update any forward-looking statements to reflect events or circumstances after the date hereof.

Item 9.01 Financial Statements and Exhibits.

(d) Exhibits

Exhibit Number	Description
99.1	Presentation, dated as of January 2022
99.2	Joint Press Release, dated as of January 10, 2022.
104	Cover Page Interactive Data File (the cover page XBRL tags are embedded within the inline XBRL document).

SIGNATURE

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 hereunto duly authorized.

STOKE THERAPEUTICS, INC.

Date: January 10, 2022

By: /s/ Stephen J. Tulipano
Stephen J. Tulipano
Chief Financial Officer

Exhibit 99.1

Stoke Therapeutics

NASDAQ: STOK

Edward M. Kaye, M.D.
Chief Executive Officer

40th Annual J.P. Morgan Healthcare Conference
January 10, 2022

Disclaimer

This presentation has been prepared by Stoke Therapeutics, Inc. ("Stoke" or "our") for informational purposes only and not for any other purpose. Nothing contained in this presentation is, or should be construed as, a recommendation, promise or representation by the presenter or Stoke or any officer, director, employee, agent or advisor of Stoke. This presentation does not purport to be all-inclusive or to contain all of the information you may desire. Information provided in this presentation speaks only as of the date hereof. Stoke assumes no obligation to publicly update any information or forward-looking statement, whether written or oral, that may be made from time to time, whether as a result of new information, future developments, subsequent events, or circumstances after the date hereof, or to reflect the occurrence of unanticipated events.

This presentation contains "forward-looking" statements within the meaning of the "safe harbor" provisions of the Private Securities Litigation Reform Act of 1995, including, but not limited to: the ability of our TANGO platform to design medicines to increase protein production and the expected benefits thereof; expectations regarding our aspirations to execute in the clinic with STK-001, advance to the clinic with STK-002, and expand our pipeline through internal discovery and collaboration; the ability of STK-001 to treat the underlying causes of Dravet syndrome and reduce seizures; the ability of STK-002 to treat the underlying causes of Autosomal Dominant Optic Atrophy (ADOA); the preclinical data and study results regarding OPA1; our future operating results, financial position and liquidity; our expectations about timing and execution of anticipated milestones, responses to regulatory authorities, expected nomination of future product candidates and timing thereof; our expectations, plans, aspirations and goals, including those related to the goals of our collaboration with Acadia; and our preliminary cash, cash equivalents, marketable securities and restricted cash and shares outstanding as of December 31, 2021. These forward-looking statements may be accompanied by such words as "aim," "anticipate," "believe," "could," "estimate," "expect," "forecast," "goal," "intend," "may," "might," "plan," "potential," "possible," "will," "would," and other words and terms of similar meaning. These forward-looking statements involve risks and uncertainties, as well as assumptions, which, if they do not fully materialize or prove incorrect, could cause our results to differ materially from those expressed or implied by such statements, including: our ability to develop, obtain regulatory approval for and commercialize STK-001, STK-002, and future product candidates, including any future product candidates nominated for SYNGAP1 or MECP2; the timing and results of preclinical studies and clinical trials; the risk that positive results in a clinical trial may not be replicated in subsequent trials or success in early stage clinical trials may not be predictive of results in later stage clinical trials; risks associated with clinical trials, including our ability to adequately manage clinical activities, unexpected concerns that may arise from additional data or analysis obtained during clinical trials, regulatory authorities may require additional information or further studies, or may fail to approve or may delay approval of our drug candidates; the occurrence of adverse safety events; failure to protect and enforce our intellectual property and other proprietary rights; failure to successfully execute or realize the anticipated benefits of our strategic and growth initiatives, including our collaboration with Acadia; risks relating to technology failures or breaches; our dependence on collaborators, including Acadia, and other third parties for the development, regulatory approval, and commercialization of products and other aspects of our business, which are outside of our full control; 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; risks associated with current and potential future healthcare reforms; risks relating to attracting and retaining key personnel; failure to comply with legal and regulatory requirements; risks relating to access to capital and credit markets; environmental risks; risks relating to the use of social media for our business; and the other risks and uncertainties that are described in the Risk Factors section of our most recent annual or quarterly report and in other reports we have filed with the U.S. Securities and Exchange Commission. These statements are based on our current beliefs and expectations and speak only as of the date of this presentation. We do not undertake any obligation to publicly update any forward-looking statements.

By attending or receiving this presentation you acknowledge that you are cautioned not to place undue reliance on these forward-looking statements, which speak only as of the date such statements are made; you will be solely responsible for your own assessment of the market and our market position; and that you will conduct your own analysis and be solely responsible for forming your own view of the potential future performance of Stoke.

STOKE THERAPEUTICS

Boldly Restoring Genetic Health

Addressing the underlying cause of severe diseases by upregulating protein expression with RNA-based medicines

Execute in the clinic with STK-001, the first potential disease-modifying approach for the treatment of Dravet syndrome

Advance to the clinic with STK-002, the first potential disease-modifying approach for the treatment of Autosomal Dominant Optic Atrophy (ADOA)

Expand pipeline through internal discovery and collaboration

A Differentiated Platform for the Discovery and Development of Novel RNA-Based Medicines

Proprietary RNA therapeutics platform (TANGO)

Targets pre-mRNA splicing to restore target protein to near-normal levels

Disease-modifying approach

We aim to address the underlying cause of severe diseases

Clinical stage with emerging pipeline

Phase 1/2a studies ongoing with STK-001 for Dravet syndrome (DS). Preclinical development initiated for STK-002 for autosomal dominant optic atrophy (ADOA)

Broad therapeutic potential

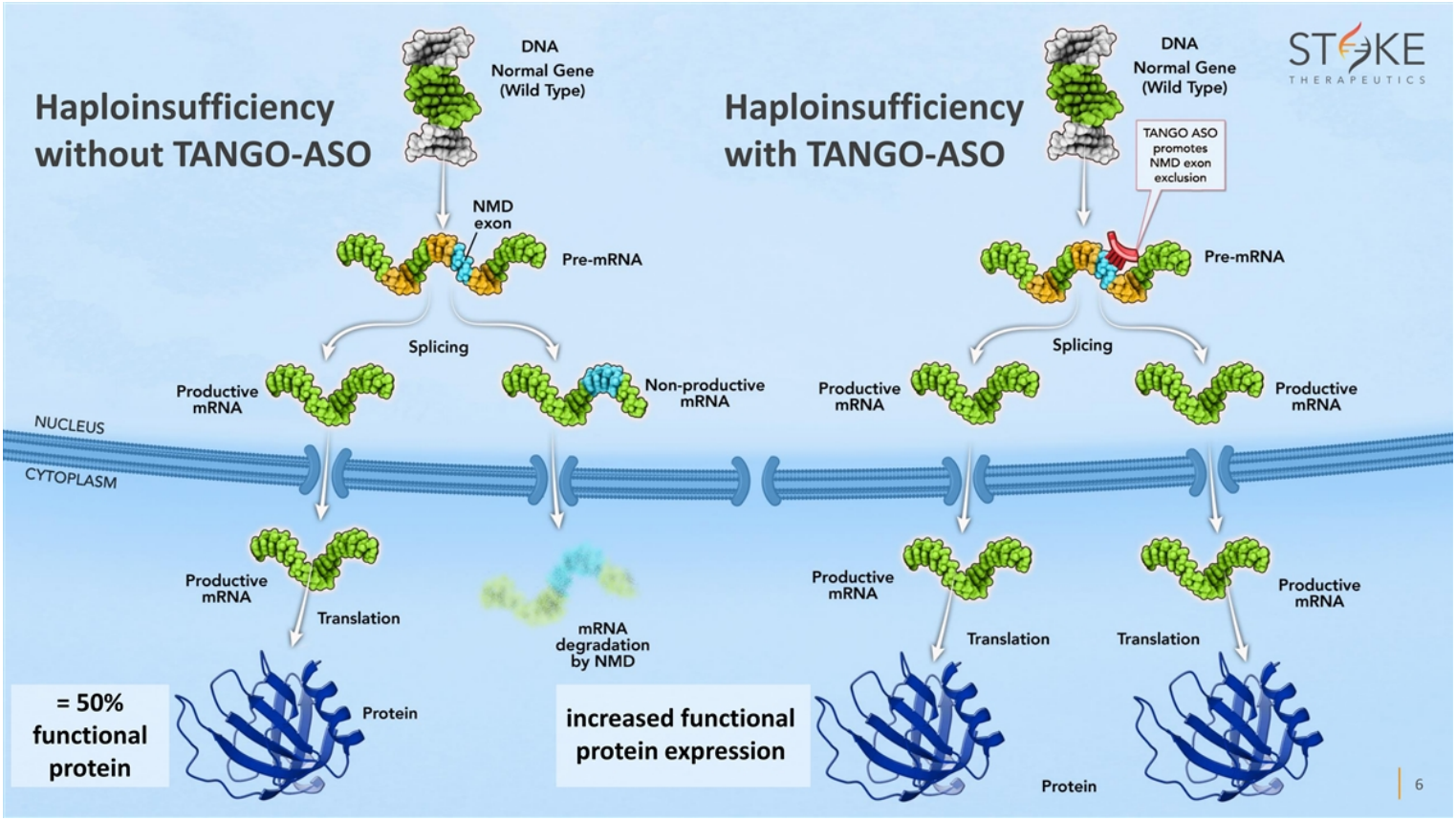
~1,200 monogenic disease genes and ~6,500 additional genes with TANGO target signatures

Source: Lim, K.H., Han, Z., Jeon, H.Y. et al. Antisense oligonucleotide modulation of non-productive alternative splicing upregulates gene expression. Nat Commun 11, 3501 (2020).

Targeted Augmentation of Nuclear Gene Output

Our compounds aim to restore protein levels by increasing protein production from the functional copy of a gene and:

- Selectively boost expression only in tissues where the protein is normally expressed
- Offer one drug for diseases caused by many different loss-of-function mutations
- Apply to genes of diverse size: can be used to address small or large gene targets


Haploinsufficiency without TANGO-ASO
DNA
Normal Gene (Wild Type)
NMD exon
Pre-mRNA
Splicing
Productive mRNA
Non-productive mRNA
NUCLEUS
CYTOPLASM
Productive mRNA
Translation
mRNA degradation by NMD
Protein
= 50% functional protein
Haploinsufficiency with TANGO-ASO
DNA
Normal Gene (Wild Type)
TANGO ASO promotes NMD exon exclusion
Pre-mRNA
Splicing
Productive mRNA
Productive mRNA
Productive mRNA
Translation
Translation
Protein
increased functional protein expression
STOKE THERAPEUTICS

2021 Execution

1H2021	Initiated Swallowtail Open Label Extension (OLE) study of STK-001 ✓
2H2021	Initiated multiple ascending dose (MAD) study of STK-001 (MONARCH) ✓
3Q2021	Reported preliminary safety, PK, and CSF data (SAD portion of MONARCH) ✓
2H2021	Initiated (MAD) study of STK-001 in the U.K. (ADMIRAL) ✓
2H2021	Initiated ADOA natural history data collection ✓
YE2021	Identified clinical candidate for the treatment of ADOA ✓

Dravet Syndrome: A Severe, Progressive Genetic Epilepsy

85%
of cases caused by a **HAPLOINSUFFICIENCY** of the *SCN1A* gene

RESULTING in

50%
$Na_V1.1$ protein expression

1 out of 16,000
babies are born with Dravet syndrome

Up to 20%
of children and adolescents with Dravet syndrome die before adulthood, due to SUDEP[1], prolonged seizures, seizure-related accidents or infections

Seizures are not adequately controlled in **90%** of people with Dravet syndrome

~35,000
people affected in the U.S., Canada, Japan, Germany, France and the UK

Dravet syndrome is not concentrated in a particular geographic area or ethnic group

[1] Sudden Unexpected Death in Epilepsy

Sources: *2018 Health Advances Report*; Djémié et al., *Molecular Genetics & Genomic Medicine*, 2016; Lagae et al., *Developmental Medicine & Child Neurology*, 2017; Nabbout et al., *Orphanet Journal of Rare Diseases*, 2013

No Approved Disease-Modifying Therapies for Dravet Syndrome

Non-Seizure Comorbidities of Dravet Syndrome Are Not Addressed by Current Therapies

- Intellectual disability
- Developmental delays
- Movement and balance issues
- Language and speech disturbances
- Growth defects
- Sleep abnormalities
- Disruptions of the autonomic nervous system
- Mood disorders

Dravet syndrome is classified as a developmental and epileptic encephalopathy due to the developmental delays and cognitive impairment associated with the disease

Non-Seizure Comorbidities of DS are Progressive and Measurable

Gap in overall intellectual development and adaptive function between patients and neurotypical children appears to widen with age

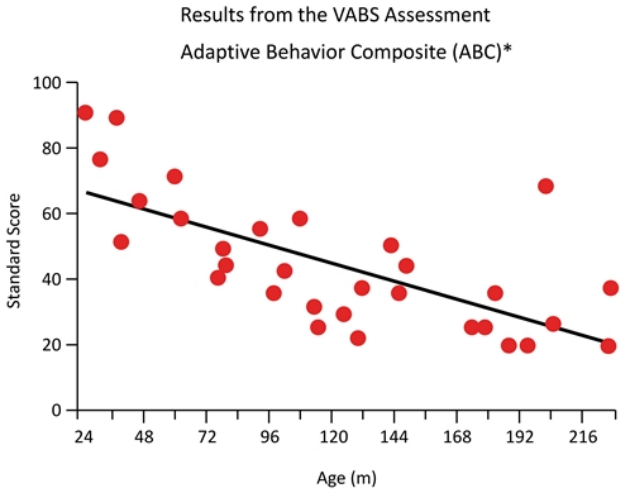
BUTTERFLY

An observational study of Dravet Syndrome patients

Initial findings showed:

- Validation of standard cognitive measures for use in DS patients
- Substantially decreased neurocognitive abilities despite the use of multiple anti-seizure medications
- A gap in adaptive functioning was observed in VABS* testing

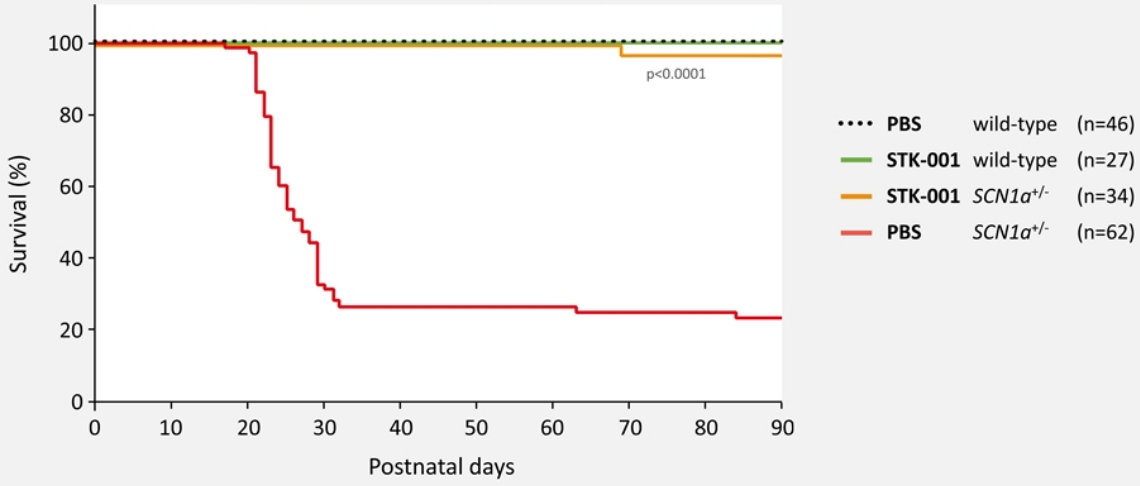
(n=36, 2-18 year-olds). Study ongoing.



* VABS = Vineland Adaptive Behavior Scales
* ABC score based on Communication, Daily Living, and Socialization domains and expressed relative to normative mean of 100
Source: BUTTERFLY: An Observational Study to Investigate Cognition and Other Non-seizure Comorbidities in Children and Adolescents with Dravet Syndrome (DS) (AES 2021).

STK-001 Significantly Reduces Premature Mortality in DS Mice After a Single Dose

Significant improvements in survival after STK-001 administration at postnatal day 2



Science Translational Medicine

Sources: Z. Han, C. Chen, A. Christiansen, S. Ji, Q. Lin, C. Anumonwo, C. Liu, S. C. Leiser, I. Aznarez, G. Liau, L. L. Isom, Antisense oligonucleotides increase Scn1a expression and reduce seizures and SUDEP incidence in a mouse model of Dravet syndrome. *Sci. Transl. Med.* 12, eaaz6100 (2020).

Preclinical Findings Support Clinical Development of STK-001

- Single dose restores $Na_V1.1$ to near-normal levels for >3 months in DS mice
- Significantly reduces mortality and seizure frequency in DS mice
- Achieves broad distribution and increases $Na_V1.1$ protein expression in NHPs
- Well-tolerated as shown in single and multiple-dose toxicology studies in NHPs

Sources: Targeted Augmentation of Nuclear Gene Output (TANGO) of SCN1A reduces seizures and rescues parvalbumin positive interneuron firing frequency in a mouse model of Dravet syndrome (AES 2020). Wengert ER, Wagley PK, Strohm SM, Reza N, Wenker IC, Gaykema RP, Christiansen A, Liau G, Patel MK. Targeted Augmentation of Nuclear Gene Output (TANGO) of Scn1a rescues parvalbumin interneuron excitability and reduces seizures in a mouse model of Dravet Syndrome. Brain Res. 2022;1775:147743. Stoke data. TANGO oligonucleotides for the treatment of Dravet Syndrome: Safety, biodistribution and pharmacology in the non-human primate (AES 2019)

Phase 1/2a Trials of STK-001 for Dravet Syndrome are Ongoing

Parallel studies in the US & UK evaluating children and adolescents ages 2 to 18 years old

	monarch	admiral
Design	Evaluation of STK-001 (up to 45mg*)	Evaluation of STK-001 (up to 70mg)
Status	• SAD: Enrollment ongoing @45mg • MAD: Enrollment and dosing ongoing @30mg	• MAD: Enrollment and dosing ongoing @30mg
Target Enrollment	~90	Up to 60
Primary Endpoint	Safety and tolerability of SAD and MAD dose levels	Safety and tolerability of MAD dose levels
	Characterize human pharmacokinetics (PK) and cerebrospinal fluid (CSF) drug exposure	
Secondary Endpoint	Change in seizure frequency, overall clinical status, and quality of life	
Open-Label Extension	Enrollment and dosing is ongoing (swallowtail)	Enrollment expected to begin in 2Q22 (Longwing)

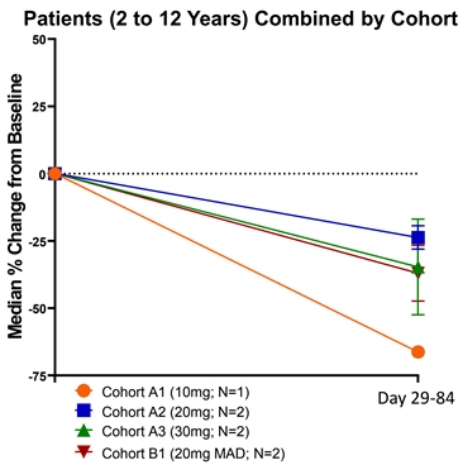
*Doses >45mg remain on FDA partial clinical hold.

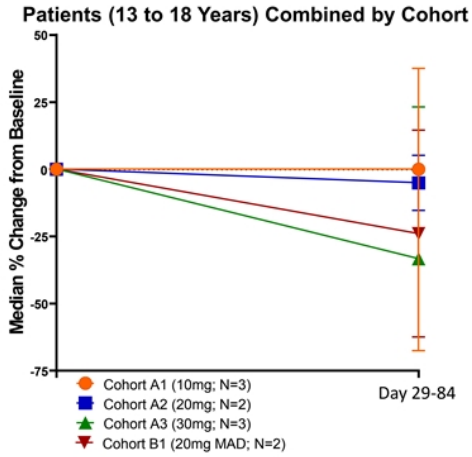
Sources: Interim Safety, PK, and CSF Exposure Data from the Phase 1/2a MONARCH Study of STK-001, an Antisense Oligonucleotide (ASO), in Children and Adolescents with Dravet Syndrome (AES 2021). ADMIRAL: A UK Study of the Safety and Pharmacokinetics of Antisense Oligonucleotide STK-001 in Children and Adolescents with Dravet Syndrome (AES 2021).

Patients Treated with STK-001 Experienced Reductions in Convulsive Seizure Frequency

70.6% (12/17) of patients including all patients ages 2-12 (n=7) experienced a reduction from baseline in convulsive seizure frequency measured from Day 29 to Day 84

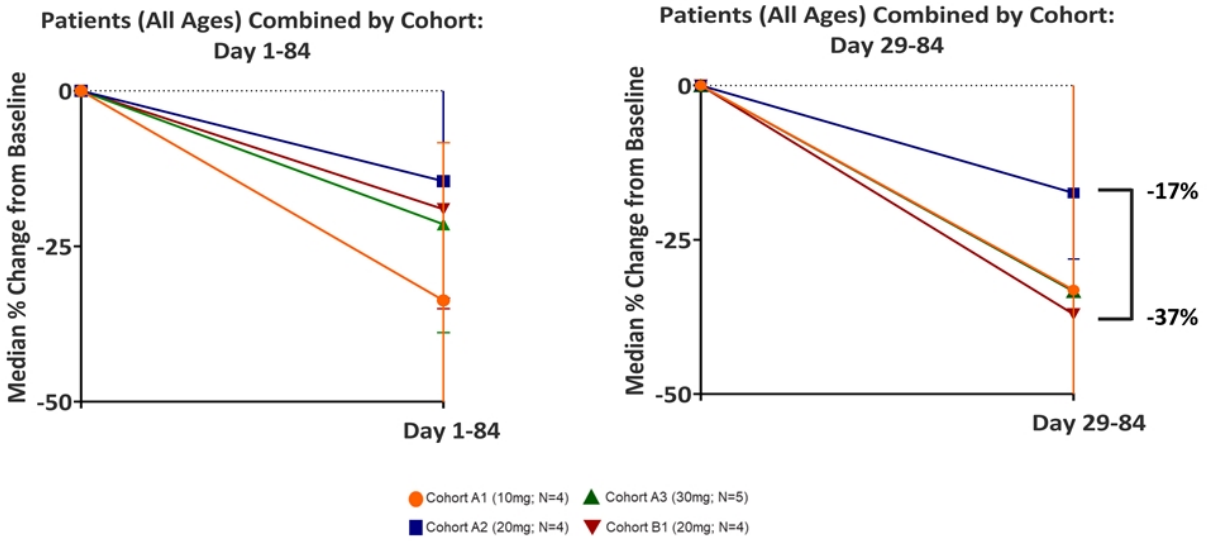
Reductions in seizure frequency were also observed among patients ages 13-18





Source: Interim Safety, PK, and CSF Exposure Data from the Phase 1/2a MONARCH Study of STK-001, an Antisense Oligonucleotide (ASO), in Children and Adolescents with Dravet Syndrome (DS) (AES 2021)

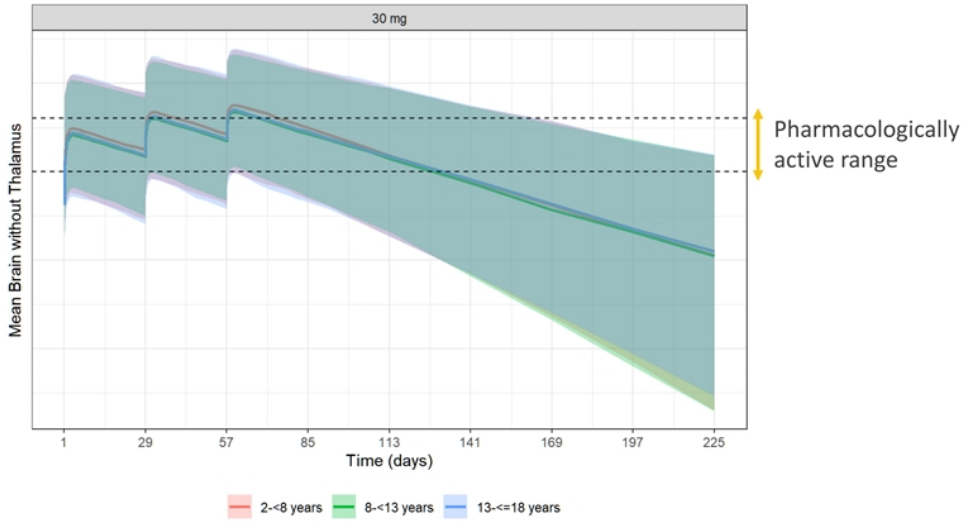
Median % Change from Baseline in Seizure Frequency More Evident >4 Weeks After Dosing



Source: Interim Safety, PK, and CSF Exposure Data from the Phase 1/2a MONARCH Study of STK-001, an Antisense Oligonucleotide (ASO), in Children and Adolescents with Dravet Syndrome (DS) (AES 2021)

3 Monthly 30mg Doses of STK-001 Projected to Achieve Pharmacologically Active Brain Levels in >95% of Patients

Plasma and CSF exposure data from MONARCH can be used to predict STK-001 brain levels in patients



Pharmacologic effect likely lasts beyond timepoint when STK-001 brain concentration falls below minimum level

Source: Interim Safety, PK, and CSF Exposure Data from the Phase 1/2a MONARCH Study of STK-001, an Antisense Oligonucleotide (ASO), in Children and Adolescents with Dravet Syndrome (DS) (AES 2021)

STK-001 Has Potential to Address the Genetic Cause of Dravet Syndrome (DS)

Summary of Ph1/2a MONARCH Interim Data

- Single doses up to 30mg and three 20mg doses were well tolerated with no safety concerns related to study drug ✓
- Plasma and CSF data from MONARCH correlated well with model and likely predict STK-001 brain levels in patients ✓
- Trend toward seizure reduction observed in DS patients following dosing of STK-001 ✓
- 3 monthly doses (30mg) predicted to achieve pharmacological active brain levels in >95% of patients ✓

Preliminary clinical data from multiple 30mg doses of STK-001 expected in the second half of 2022

Autosomal Dominant Optic Atrophy (ADOA): A Severe, Progressive Optic Nerve Disorder

65-90%
of cases caused by mutations in one allele of the *OPA1* gene, most of which lead to a **HAPLOINSUFFICIENCY**

RESULTING in

50%
OPA1 protein expression and disease manifestation

1 out of 30,000
people are affected globally with a higher incidence of ~1 out of 10,000 in Denmark due to a founder effect

>400
Different *OPA1* mutations reported in ADOA patients

Up to 46%
of patients are registered legally blind

80%
of patients are symptomatic by age 10

~18,000
people affected in the U.S., Canada, Japan, Germany, France and the UK

Sources: Yu-Wai-Man P et al. *Ophthalmology*, 2010; Yu-Wai-Man P, Chinnery PF. *Ophthalmology*, 2013; P. Amati-Bonneau P et al. *The International Journal of Biochemistry & Cell Biology*, 2009; Lenaers G, Hamel C, Delettre C, et al. *Orphanet J Rare Dis*, 2012; Chun BY and Rizzo JF III. *Curr Opin Ophthalmol*, 2016; Le Roux B, Lenaers G, Zanlonghi X et al. *Orphanet J Rare Dis*, 2019; "What is ADOA?" Autosomal Dominant Optic Atrophy Association. Accessed May 6, 2020, from https://www.adoaa.org/what-is-adoa;

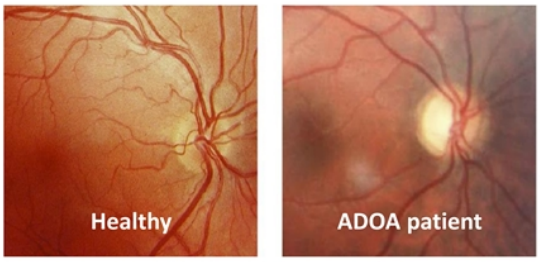
No Approved Disease-Modifying Therapies for ADOA

STOKE THERAPEUTICS

Healthy Vision

Simulation of Optic Neuropathy

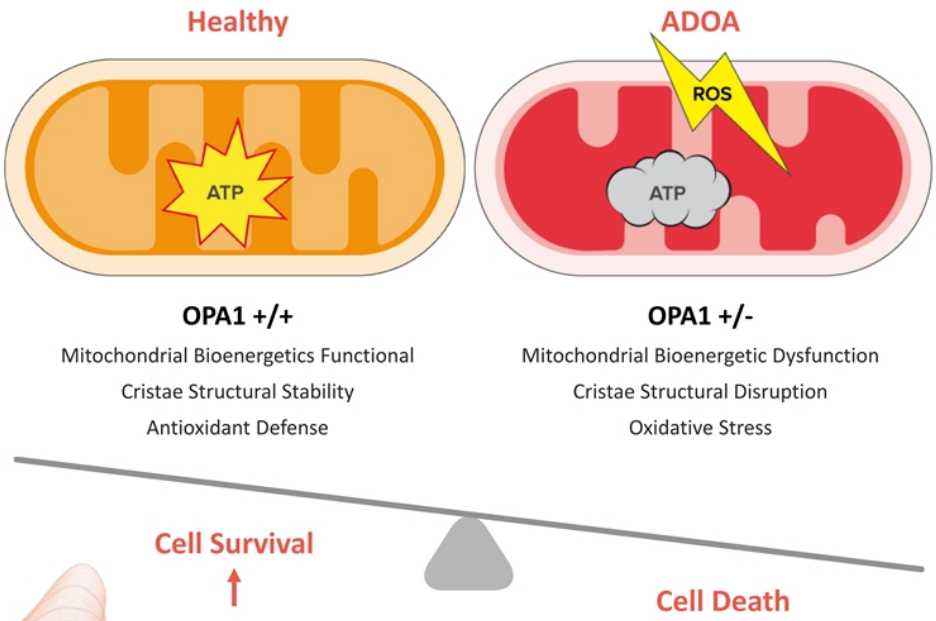
- Most common inherited optic nerve disorder
- Leads to central field defects and reduced color vision in both eyes
- Severity can vary; rate of vision loss difficult to predict
- Supportive services and low-vision aids are offered for patients



Sources: Yu-Wai-Man P et al. *Ophthalmology*, 2010; Yu-Wai-Man P, Chinnery PF. *Ophthalmology*, 2013;
Lenaers G, Hamel C, Delettre C, et al. *Orphanet J Rare Dis*, 2012; Chun BY and Rizzo JF III. *Curr Opin Ophthalmol*, 2016
Image of child sourced from ICR, Ophthalmology Center Barcelona. Accessed Jan. 8, 2021 from https://icrcat.com/en/eye-conditions/leber-hereditary-optic-neuropathy/ Credit: Lhon Eye Society Sweden. Image shown depicts Leber Hereditary Optic Neuropathy, which presents visual effects similar to ADOA.

OPA1 is Critical for Normal Mitochondrial Function and Cellular Metabolism

- Retinal ganglion cells have very high energy (ATP) requirements
- Impaired mitochondrial function leads to cell death
- OPA1 is critical for mitochondrial function and energy production

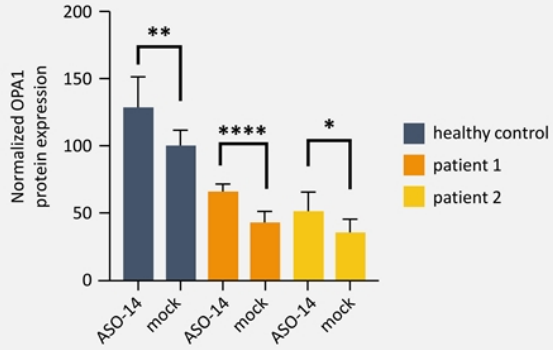


* ROS = Reactive Oxygen Species

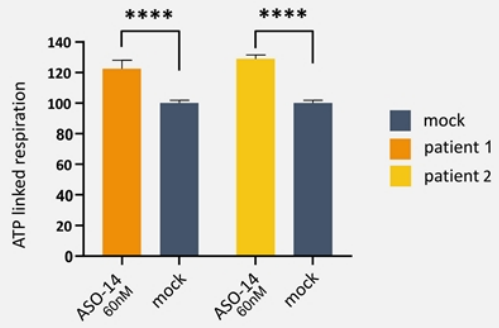
TANGO ASO Increases OPA1 Protein and ATP Linked Mitochondrial Respiration in ADOA Patient Cells

STOKE THERAPEUTICS

ASO treatment increased OPA1 protein levels in OPA1 deficient ADOA patient cells



ASO treatment increased ATP linked respiration in OPA1 deficient ADOA patient cells



Source (left graph): Stoke data
Source (right graph): Venkatesh A, et al. Antisense oligonucleotide mediated increase in OPA1 improves mitochondrial function in fibroblasts derived from patients with autosomal dominant optic atrophy (ADOA). Presented at The Association for Research in Vision and Ophthalmology; May 1-7, 2021.

TANGO ASOs Have the Potential to Address the Genetic Cause of ADOA

Summary of Key Preclinical Data

- Increase OPA1 protein and ATP linked respiration in ADOA patient cells ✓
- Result in dose-dependent increases in OPA1 protein expression in rabbit retina ✓
- Were well tolerated for up to 29 days after intravitreal injection in rabbit ✓

Preclinical toxicology studies ongoing in 2022 to support future clinical trials for STK-002

Sources: Venkatesh A, et al. Antisense oligonucleotide mediated increase in OPA1 improves mitochondrial function in fibroblasts derived from patients with autosomal dominant optic atrophy (ADOA). Presented at The Association for Research in Vision and Ophthalmology; May 1-7, 2021.Venkatesh A, et al. Antisense oligonucleotide mediated increase of OPA1 expression using TANGO technology for treatment of autosomal dominant optic atrophy. Presented at The Association for Research in Vision and Ophthalmology; May 3-7, 2020; Baltimore, MD.

Collaboration with Acadia Pharmaceuticals to Pursue RNA-Based Treatments for Severe & Rare Genetic Neurodevelopmental Diseases

Collaboration leverages Stoke's proprietary TANGO research platform and Acadia's expertise in neurology drug development and commercialization

3 targets focused on severe and rare genetic neurodevelopmental diseases of the central nervous system

- Acadia receives exclusive worldwide licenses for:
  - Rett syndrome (*MECP2*)
  - Undisclosed neurodevelopmental target
- 50:50 co-development co-commercialization of SYNGAP1

Stoke receives a $60M upfront payment and potential milestones up to $907M as well as royalties on future sales

- Acadia fully funds the research and preclinical development activities for Rett syndrome (MECP2) and undisclosed neurodevelopmental program
- Share 50/50 in all world-wide costs and future profits for SYNGAP1 program

Rett Syndrome: A Severe, Debilitating Neurological Disorder

~33%

of cases caused by hypomorphic mutations of the *MECP2* gene[1]

RESULTING in

Partial loss of function of the MeCP2 protein

1 out of 10,000 to 15,000 females are born with Rett syndrome[2]

Period of rapid decline typically begins between

6 to 18

months[4]

Symptoms include[3]:

- **Loss of purposeful hand use**
- **Involuntary hand movements such as handwringing**
- **Loss of speech**
- **Loss of mobility or gait disturbances**

60-80% of patients have **epilepsy**[4]

Note: All seizure types have been reported in Rett syndrome. Complex partial and generalized tonic-clonic are the most common
Sources: [1] RettBase (http://mecp2.chw.edu.au/); GnomAD (https://gnomad.broadinstitute.org); NOMAD; [2] National Institutes of Health – National Institute of Neurological Disorders and Stroke; [3] International Rett Syndrome Foundation; [4] Operta et al., Brain Behav 2019

SYNGAP1 Syndrome: A Severe Intellectual Disability / Developmental and Epileptic Encephalopathy (ID/DEE)

>80%
of cases caused by a **HAPLOINSUFFICIENCY** of the *SYNGAP1* gene[1]

RESULTING in

50%
SynGAP protein expression

~1-2 out of 100,000 children are born with SYNGAP1-ID/DEE

1-2%
of all **intellectual disability** cases[2]

84%
of patients have **generalized epilepsy**[3]

100%
of patients have **developmental delay** or **intellectual disability**[3]

~50%
of patients have **autism and other behavioral abnormalities**[3]

Sources: [1] Parker et al., *American Journal of Medical Genetics*, 2015; Jimenez-Gomez et al., Journal of Neurodevelopmental Disorders, 2019; [2] SYNGAP1 Resource Guide, Second Edition; An Overview of SYNGAP1 Basic Biology and Clinical Description. Bridge the Gap SYNGAP (now SYNGAP1 Foundation); SynGAP Research Fund; [3] SYNGAP1-Related Intellectual Disability: https://www.ncbi.nlm.nih.gov/books/NBK537721/#_syngap1-id_Clinical_Characteristics_

Investing In Our Pipeline

PROGRAM	TARGET	DISCOVERY & PRECLINICAL	PHASE 1/2	PHASE 3	PARTNER
Central Nervous System					
Dravet Syndrome	*SCN1A*	STK-001			100% Stoke Global
SYNGAP1 Syndrome	*SYNGAP1*				Stoke: Acadia 50:50
Rett Syndrome	*MECP2*				Acadia Worldwide License
Undisclosed	*Undisclosed*				Acadia Worldwide License
Ophthalmology					
ADOA	*OPA1*	STK-002			100% Stoke Global

Our Strategy For 2022

Advance our wholly owned CNS and eye programs and expand the scope of our drug discovery efforts

Advance STK-001 for Dravet Syndrome

- Additional clinical data on STK-001 (30mg MAD) anticipated in 2H22
- Initiate dosing >30mg in MONARCH and ADMIRAL

Advance STK-002 for ADOA

- Conduct preclinical toxicology studies to support future clinical trials for STK-002
- Begin enrollment in prospective ADOA natural history study
- Present additional preclinical data for STK-002 at scientific forum

Develop & Expand Pipeline

- Continue discovery efforts to identify new targets
- Execute on collaboration with Acadia

Current Liquidity Including Upfront from Acadia Anticipated to Fund Operations into the Second Half of 2024

$220.4M*

Cash, Cash Equivalents,

Marketable Securities, and Restricted Cash

(unaudited) as of 12/31/2021

36.9M

Common Shares Outstanding

(unaudited) as of 12/31/2021

*Does not include the $60 Million Upfront from Acadia

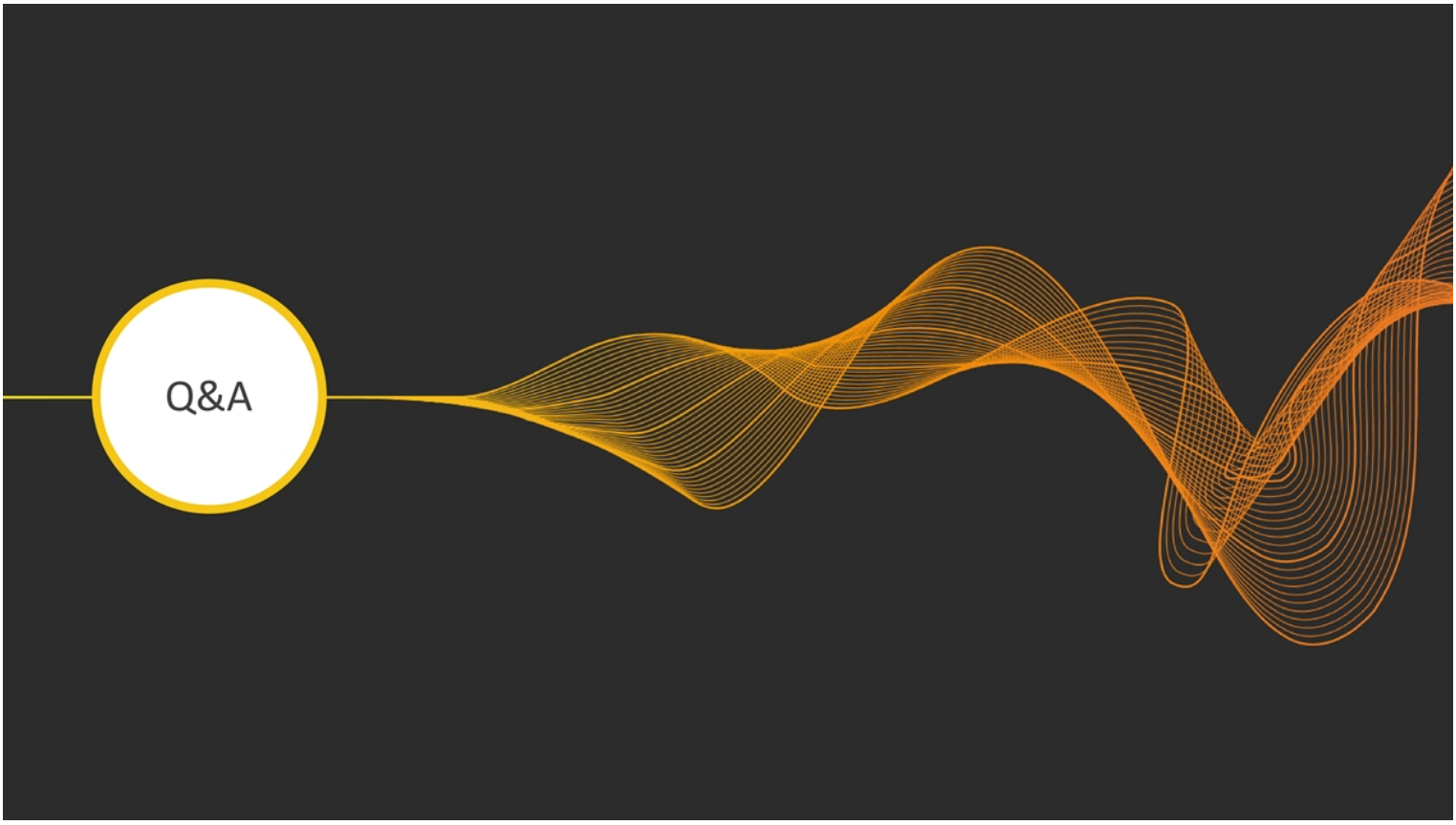

Q&A

Exhibit 99.2

Acadia Pharmaceuticals and Stoke Therapeutics Announce Collaboration to Pursue Multiple RNA-based Treatments for Severe and Rare Genetic Neurodevelopmental Diseases

- Establishes co-development and co-commercialization agreement for Stoke's SYNGAP1 preclinical program

- Acadia receives exclusive worldwide licenses for two additional preclinical programs: Rett syndrome (MECP2) and undisclosed neurodevelopmental target

- Combines Stoke's TANGO research platform with Acadia's expertise in neurology drug development and commercialization

- Stoke receives a $60 million upfront payment and potential milestone payments of up to $907 million and royalties on future sales

SAN DIEGO, CA and BEDFORD, MA, January 10, 2022 – Acadia Pharmaceuticals Inc. (Nasdaq: ACAD) and Stoke Therapeutics, Inc. (Nasdaq: STOK) announced today that the companies have entered a collaboration to discover, develop and commercialize novel RNA-based medicines for the potential treatment of severe and rare genetic neurodevelopmental diseases of the central nervous system (CNS). The collaboration includes SYNGAP1 syndrome, Rett syndrome (*MECP2*), and an undisclosed neurodevelopmental target of mutual interest.

"Stoke's RNA-based approach to upregulating healthy proteins offers very exciting new possibilities for the treatment of rare, neurodevelopmental diseases like Rett syndrome," said Steve Davis, Chief Executive Officer of Acadia Pharmaceuticals. "Combining Stoke's capabilities with Acadia's extensive expertise in neuroscience drug development and commercialization enables us to push harder and faster in exploring some of the new frontiers in rare central nervous system disorders. We are excited to have the opportunity to further build our Rett syndrome franchise and pursue treatments in SYNGAP1 syndrome and other neurodevelopmental disorders."

"Rett syndrome and SYNGAP1 syndrome are two severe, intractable diseases of the central nervous system and both are associated with developmental delays, motor function loss, autism, seizures and other comorbidities that impact quality of life for patients and their families," said Edward M. Kaye, M.D., Chief Executive Officer of Stoke Therapeutics. "Acadia shares our deep commitment to improving outcomes for people living with neurodevelopmental disorders. We believe their late-stage development and commercialization capabilities, in addition to their neuroscience expertise, complement our discovery research efforts and clinical learnings from our work in Dravet syndrome. Together, we believe we have a significant opportunity to improve treatment options by delivering new disease-modifying medicines to people who need them."

Terms of Collaboration

Under the terms of the agreement, Stoke will receive an upfront payment of $60 million from Acadia and is eligible to receive up to $907 million in milestones as well as royalties on future sales.

For the SYNGAP1 program, the two companies will jointly share global research, development and commercialization responsibilities and share 50/50 in all worldwide costs and future profits. In addition, Stoke is eligible to receive potential development, regulatory, first commercial sales and sales milestones.

For the Rett syndrome *(MECP2*) and the undisclosed neurodevelopmental program, Stoke will lead research and pre-clinical development activities, while Acadia will lead clinical development and commercialization activities. Acadia will fully fund the research and pre-clinical development activities related to these two targets and Stoke is eligible to receive potential development, regulatory, first commercial sales and sales milestones as well as tiered royalty payments on worldwide sales starting in the mid-single digit range and escalating to the mid-teens based on revenue levels.

About SYNGAP1 Syndrome

SYNGAP1 syndrome is a rare neurological disorder characterized by moderate to severe intellectual disability that is evident in early childhood. Mutations in the *SYNGAP1* gene (which produces the SynGAP protein) were first identified in 2009 and since then, an increasing number of children with SYNGAP1 syndrome have been identified. Normal levels of SynGAP protein are essential for proper brain function and development. Mutations in the *SYNGAP1* gene also play an important role in the development of epileptic encephalopathies (DEEs). The severity and onset of symptoms can vary from patient to patient. SYNGAP1 syndrome is characterized by developmental delay or intellectual disability, generalized epilepsy, and autism spectrum disorder (ASD) and other behavioral abnormalities. More than 80% of cases of SYNGAP1 syndrome are caused by a haploinsufficiency of the *SYNGAP1* gene. SYNGAP1 syndrome is estimated to account for 1% to 2% of all intellectual disability cases. There are currently no approved treatments for SYNGAP1 syndrome.

About Rett Syndrome

Rett syndrome is a rare, debilitating neurological disorder that occurs primarily in females following apparently normal development for the first six months of life. Rett syndrome is often misdiagnosed as autism, cerebral palsy, or non-specific developmental delay. Rett syndrome is caused by mutations on the X chromosome on a gene called *MECP2.* There are more than 200 different mutations found on the *MECP2* gene that interfere with its ability to generate a normal gene product. Rett syndrome occurs worldwide in approximately one of every 10,000 to 15,000 female births and in the United States impacts 6,000 to 9,000 patients. Rett syndrome causes problems in brain function that are responsible for cognitive, sensory, emotional, motor and autonomic function. Typically, with symptoms presenting between 6 to 18 months of age, patients experience a period of rapid decline with loss of purposeful hand use (fine motor skills), development of hand stereotypies, absent or impaired mobility (gross motor skills), loss of communication skills (including eye contact) and inability to independently conduct activities of daily living. Symptoms also include seizures, disorganized breathing patterns, an abnormal side-to-side curvature of the spine (scoliosis), and sleep disturbances. Currently, there are no FDA-approved medicines for the treatment of Rett syndrome.

About TANGO

TANGO (Targeted Augmentation of Nuclear Gene Output) is Stoke's proprietary research platform. Stoke's initial application for this technology are diseases in which one copy of a gene functions normally and the other is mutated, also called haploinsufficiencies. In these cases, the mutated gene does not produce its share of protein, resulting in disease. Using the TANGO approach and a deep understanding of RNA science, Stoke researchers design antisense oligonucleotides (ASOs) that bind to pre-mRNA and help the functional (or wild-type) genes produce more protein. TANGO aims to restore missing proteins by increasing – or stoking – protein output from healthy genes, thus compensating for the mutant copy of the gene.

About Stoke Therapeutics

Stoke Therapeutics (Nasdaq: STOK), is a biotechnology company dedicated to addressing the underlying cause of severe diseases by upregulating protein expression with RNA-based medicines. Using Stoke's proprietary TANGO (Targeted Augmentation of Nuclear Gene Output) approach, Stoke is developing antisense oligonucleotides (ASOs) to selectively restore protein levels. Stoke's first compound, STK-001, is in clinical testing for the treatment of Dravet syndrome, a severe and progressive genetic epilepsy. Dravet syndrome is one of many diseases caused by a haploinsufficiency, in which a loss of ~50% of normal protein levels leads to disease. Stoke is pursuing treatment for a second haploinsufficient disease, autosomal dominant optic atrophy (ADOA), the most common inherited optic nerve disorder. Stoke's initial focus is haploinsufficiencies and diseases of the central nervous system and the eye, although proof of concept has been demonstrated in other organs, tissues, and systems, supporting its belief in the broad potential for its proprietary approach. Stoke is headquartered in Bedford, Massachusetts with offices in Cambridge, Massachusetts. For more information, visit https://www.stoketherapeutics.com/ or follow Stoke on Twitter at @StokeTx.

About Acadia Pharmaceuticals

Acadia is advancing breakthroughs in neuroscience to elevate life. For more than 25 years Acadia has been working at the forefront of healthcare to bring vital solutions to people who need them most. Acadia developed and commercialized the first and only approved therapy for hallucinations and delusions associated with Parkinson's disease psychosis. Acadia's late-stage development efforts are focused on treating psychosis in patients with dementia, the negative symptoms of schizophrenia and Rett syndrome. Acadia's early-stage development efforts are focused on novel approaches to pain management, cognition and neuropsychiatric symptoms in central nervous system disorders. For more information, visit us at www.acadia-pharm.com and follow us on LinkedIn and Twitter.

Stoke's Cautionary Note Regarding Forward-Looking Statements

This press release contains forward-looking statements within the meaning of the "safe harbor" provisions of the Private Securities Litigation Reform Act of 1995, including, but not limited to, statements regarding: receipt of upfront payments; receipt of potential milestone payments under the SYNGAP1 collaboration; receipt of potential milestones and royalty payments under the MECP2 program and the third program; the ability to develop new treatments for neurodevelopmental disorders; and expectations regarding the proposed transaction with Acadia. Statements including words such as "believe," "will," "eligible," or "potential," and any other statements in the future tense are forward-looking statements. These forward-looking statements involve risks and uncertainties, as well as assumptions, which, if they do not fully materialize or prove incorrect, could cause our results to differ materially from those expressed or implied by such forward-looking statements. Forward-looking statements are subject to risks and uncertainties that may cause the company's actual activities or results to differ significantly from those expressed in any forward-looking statement, including risks and uncertainties related to: Stoke's ability to develop and identify potential product candidates; that if Acadia were to breach or terminate the collaboration, Stoke would not obtain the anticipated financial or other benefits; the possibility that Stoke and Acadia may not be successful in their development efforts under any of the collaborations and that, even if successful, Stoke and Acadia may be unable to successfully commercialize any resulting product candidates; and other risks and uncertainties described under the heading "Risk Factors" in documents Stoke files from time to time with the Securities and Exchange Commission. These forward-looking statements speak only as of the date of this press release and Stoke undertakes no obligation to revise or update any forward-looking statements to reflect events or circumstances after the date hereof.

Acadia's Forward-Looking Statement

Statements in this press release that are not strictly historical in nature are forward-looking statements. These statements include but are not limited to statements regarding the timing of future events. These statements are only predictions based on current information and expectations and involve a number of risks and uncertainties. Actual events or results may differ materially from those projected in any of such statements due to various factors, including the risks and uncertainties inherent in drug development, approval and commercialization. For a discussion of these and other factors, please refer to Acadia's annual report on Form 10-K for the year ended December 31, 2020 as well as Acadia's subsequent filings with the Securities and Exchange Commission. You are cautioned not to place undue reliance on these forward-looking statements, which speak only as of the date hereof. This caution is made under the safe harbor provisions of the Private Securities Litigation Reform Act of 1995. All forward-looking statements are qualified in their entirety by this cautionary statement and Acadia undertakes no obligation to revise or update this press release to reflect events or circumstances after the date hereof, except as required by law.

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