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 13, 2020

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

Delaware	**001-38938**	**47-114582**
(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 7.01 Regulation FD.

Stoke Therapeutics, Inc. plans to present the presentation attached hereto as Exhibit 99.1 at the J.P. Morgan Healthcare Conference on January 14, 2020.

The information furnished with this report, including Exhibit 99.1, shall not be deemed "filed" for purposes of Section 18 of the Securities Exchange Act of 1934, 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, except as expressly set forth by specific reference in such a filing.

Item 9.01 Financial Statements and Exhibits.

(d) Exhibits

Exhibit Number	Description
99.1	Stoke Presentation

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 13, 2020

By: /s/ Robin A. Walker

Robin A. Walker
Senior Vice President, Chief Legal Officer and Chief Compliance Officer

Exhibit 99.1

Stoke Therapeutics

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

J.P. Morgan 2020 Healthcare Conference
January 14, 2020

Nasdaq: STOK

Disclaimer

This presentation has been prepared by Stoke Therapeutics, Inc. ("Stoke") 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 includes express and implied "forward-looking statements." In some cases, you can identify forward-looking statements by terms such as "anticipate," "believe," "estimate," "expect," "intend," "may," "might," "plan," "project," "target," "will," "would," "should," "could," "can," "predict," "potential," "continue," or the negative of these terms, and similar expressions intended to identify forward-looking statements. However, not all forward-looking statements contain these identifying words. These statements may relate to our strategic plans or objectives, revenues or earnings projections, other financial items, the timing, progress, and results of preclinical studies, timing and likelihood of regulatory approvals, the timing of anticipated clinical trials and data readouts, or business development plans and opportunities. By their nature, these statements are subject to numerous uncertainties, including factors beyond our control, that could cause actual results, performance or achievement to differ materially and adversely from those anticipated or implied in the statements. You should not rely upon forward-looking statements as predictions of future events. Although our management believes that the expectations reflected in our statements are reasonable, we cannot guarantee that the future results, levels of activity, performance or events and circumstances described in the forward-looking statements will be achieved or occur. Further information on potential risk factors that could affect our business and its financial results are detailed in our most recent Quarterly Report on Form 10-Q for the quarter ended September 30, 2019 filed with the Securities and Exchange Commission (SEC), and other reports as filed with the SEC. Although we believe that the expectations reflected in the forward-looking statements are reasonable, we cannot guarantee future results, levels of activity, performance, achievements or events and circumstances reflected in the forward-looking statements will occur.

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

Amplifying Science to Transform the Experience of Life

> Stoke is making a new generation of RNA-based genetic medicines that up-regulate protein expression to restore human health.

STOKE THERAPEUTICS

Foundational Elements of Stoke

Experienced Leaders in Innovation

Differentiated Platform with Broad Applicability

Focused Development Program

Strong Financial Position to Support Growth

Efficient Execution Since 2018 Launch of Stoke

2018

Stoke is Launched

- Closed $40M Series A financing
- Nominated Dravet syndrome as lead program; generated *in-vivo* proof of concept
- Completed FDA pre-IND meeting
- Closed $90M Series B financing
- Built robust intellectual property estate

2019

Stoke is Poised to Enter the Clinic

- Completed $163.3M Initial Public Offering
- Received FDA orphan drug designation for STK-001, a potential disease modifying medicine for Dravet syndrome
- Enrolled first patient in the BUTTERFLY observational study
- Presented preclinical data supporting efficacy of STK-001
- Completed IND-enabling studies for STK-001

STOKE THERAPEUTICS

Experienced Leaders in Innovation

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

Huw Nash, Ph.D.
Chief Operating Officer and Chief Business Officer

Barry Ticho, M.D., Ph.D.
Chief Medical Officer

Steve Tulipano, CPA
Chief Financial Officer

Gene Liau, Ph.D.
Executive Vice President, Head of Research and Preclinical Development

Robin Walker, J.D.
Senior Vice President, Chief Legal Officer and Chief Compliance Officer

TANGO: An RNA-Based Genetic Medicine Platform for Protein Upregulation

Stoke uses RNA science to restore missing proteins by increasing – or stoking – protein output from healthy genes.

TANGO

Targeted **A**ugmentation of **N**uclear **G**ene **O**utput

- Addresses underlying cause of disease
- Applicable to most loss-of-function mutations
- Applies equally to small or large gene targets
- Gene and tissue specific
- Controllable dose and duration
- Can address wide array of diseases
- Simple and scalable manufacturing

STOKE THERAPEUTICS

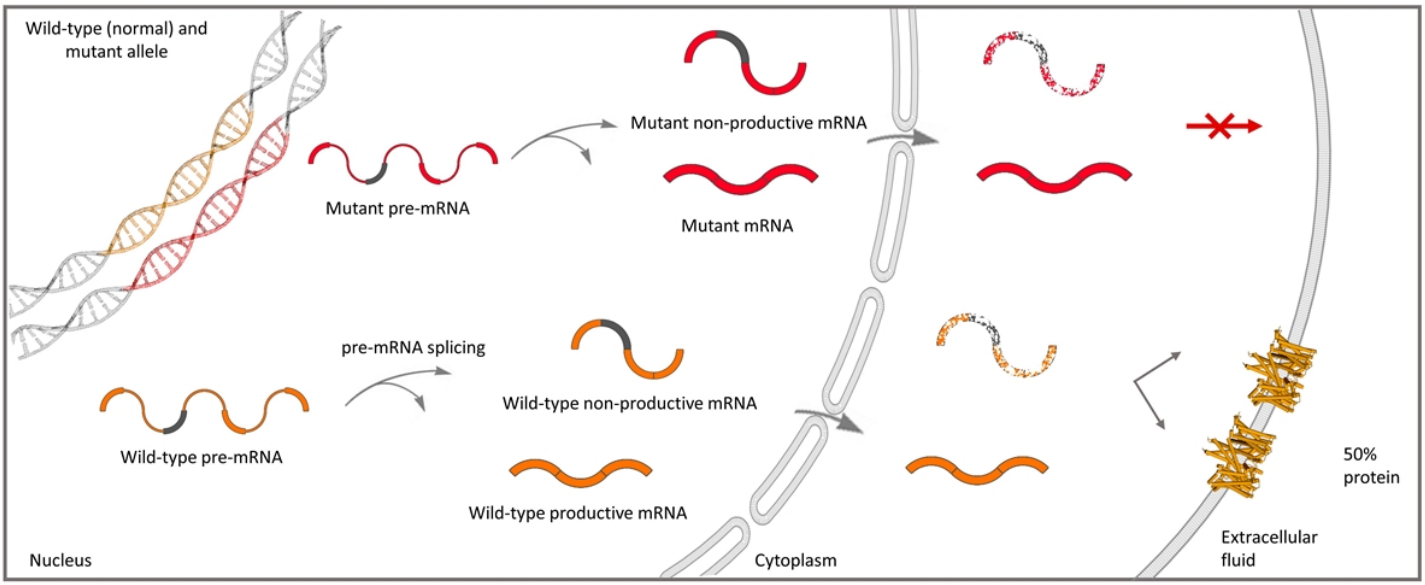
TANGO Restores Protein Levels by Stoking Output From Healthy Genes

- Stoke's ASOs bind to specific stretches of pre-mRNA to **reduce non-productive mRNA and increase productive mRNA**
- The increased levels of productive mRNA from the functional copy of the gene result in **increased protein production**
- For **haploinsufficiences**, TANGO restores the target protein to near-normal levels

STOKE THERAPEUTICS

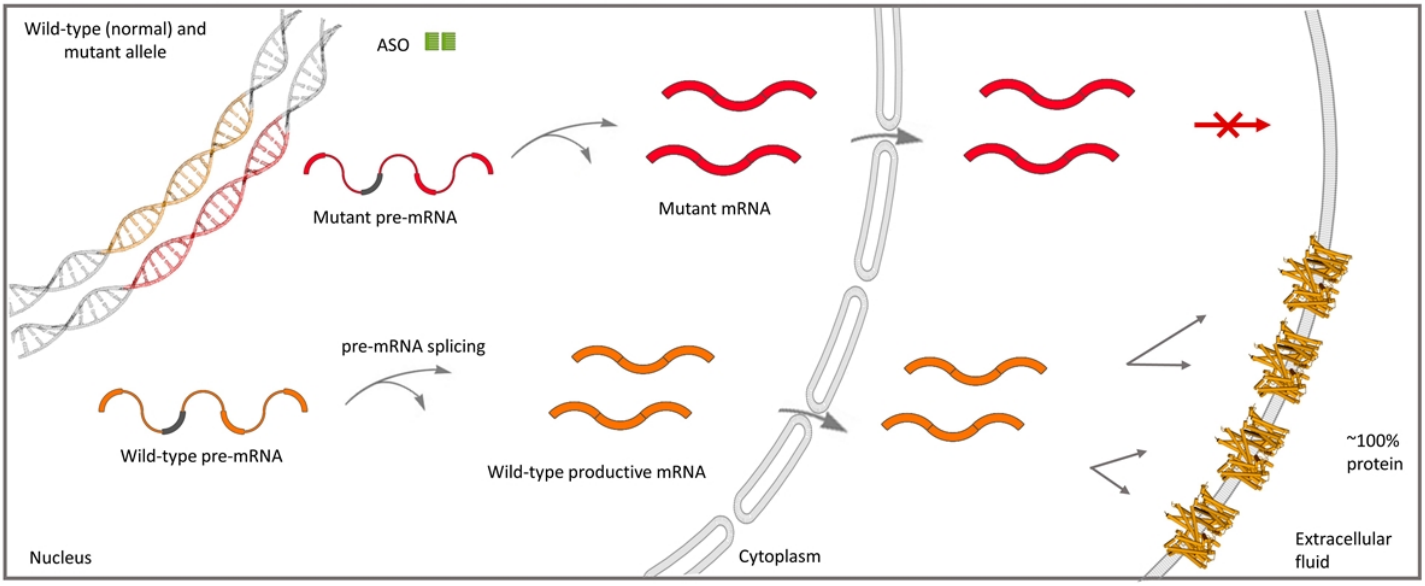
Transformative Potential of TANGO Technology for Haploinsufficiencies

TANGO mechanism for increasing protein synthesis in a prospective haploinsufficient patient.

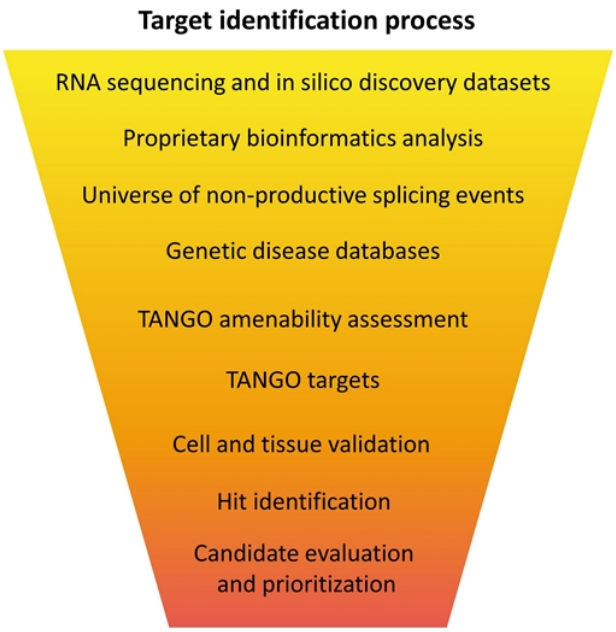


Transformative Potential of TANGO Technology for Haploinsufficiencies

TANGO mechanism for increasing protein synthesis in a prospective haploinsufficient patient.



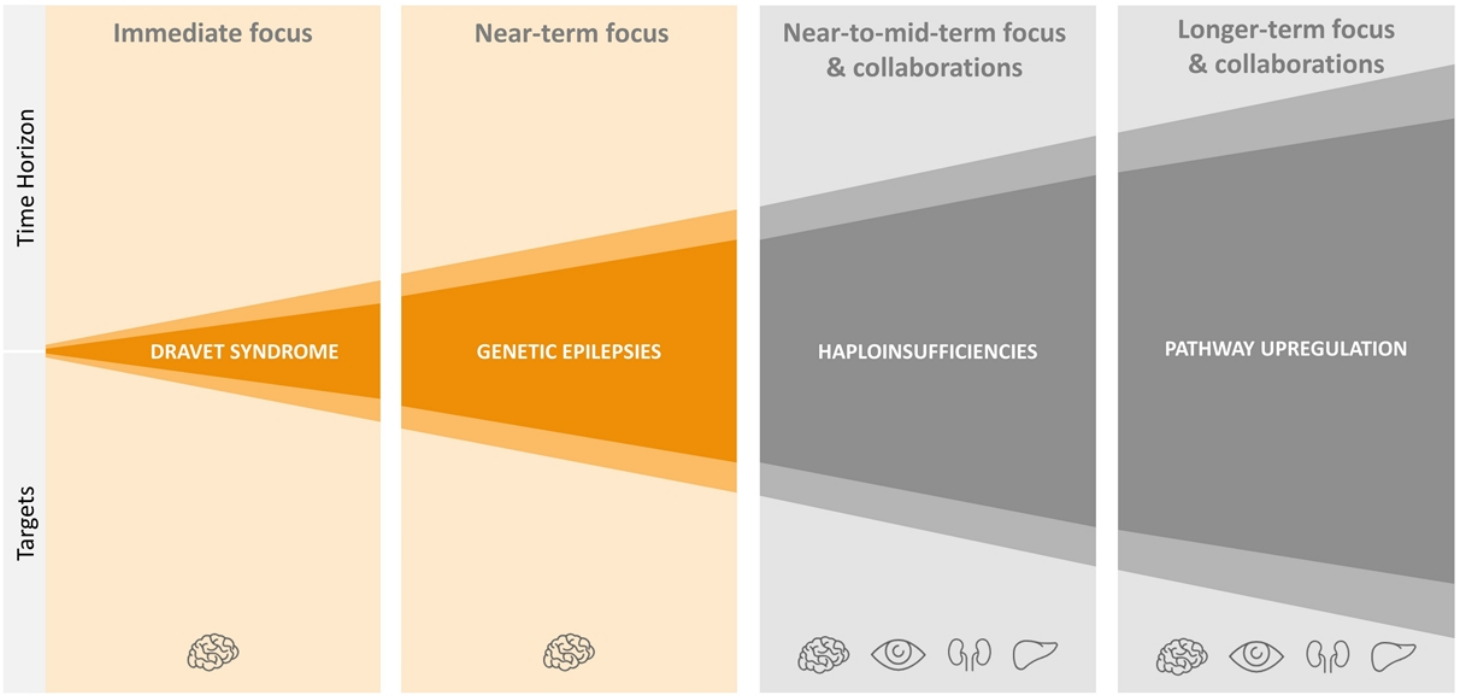
Robust Target Identification Process Utilizing Proprietary Bioinformatics



- Approximately 50% of human genes contain a TANGO signature
- Cross-referencing with genetic disease databases identifies approximately 2,900 monogenic diseases amenable to TANGO
- Enables rapid and systematic identification of clinically relevant targets

Source: Stoke data

Stoke is Initially Focused on Dravet Syndrome and Other Genetic Epilepsies



Significant Unmet Need in Genetic Epilepsies

50 million people globally affected by epilepsy

>30% of patients are refractory to medical treatment, especially those with a genetic epilepsy

Up to **50%** of patients with epilepsy have significant cognitive problems

>50% of epilepsies have an identified genetic cause and many of these are haploinsufficiencies

Diagnostic work-up of epilepsy routinely includes genetic testing for more than **180** disease associated genes

While genetic mechanisms are often well understood …

0 genetically-targeted therapies for epilepsies are available

Source: WHO 2018 fact sheet; Sirven, Cold Spring Harbor Perspectives in Medicine 2015; Pal et al., *Nature Reviews Neurology* 2010; Chen et al., *JAMA Neurology* 2018; Lagae et al., *Developmental Medicine & Child Neurology* 2017; Vlaskamp et al., *Neurology* 2019; Reddy SD et al., J Pharmacol Exp Ther 2018; NIH Genetics Home Reference; Company websites

STOKE THERAPEUTICS

Dravet Syndrome: A Severe, Progressive Genetic Epilepsy

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

results in

50% $Na_V1.1$ protein expression

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

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

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

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

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

Sources: Source: *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

STOKE THERAPEUTICS

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

Dravet is Not Limited to Seizures:

More than 90% of patients suffer from at least one non-seizure comorbidity, including

- Severe intellectual disabilities
- Severe developmental disabilities
- Motor impairment
- Speech impairment
- Autism
- Behavioral difficulties
- Sleep abnormalities

High Incidence of Premature Death:

Up to 20% of children and adolescents die before adulthood, due to:

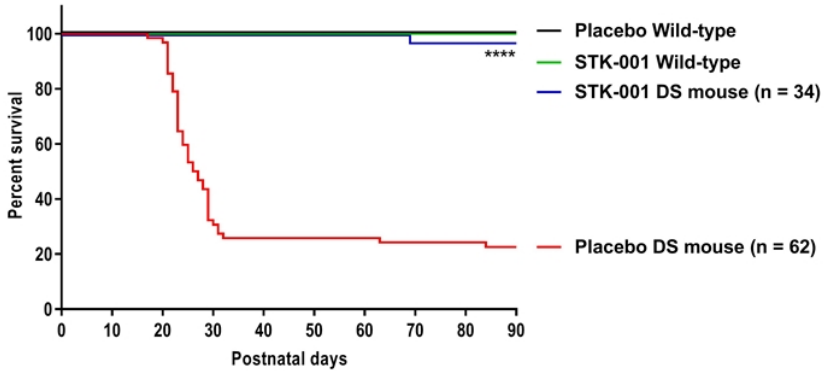
- SUDEP[1]
- Prolonged seizures
- Seizure-related accidents
- Infections

Note: [1] Sudden Unexpected Death in Epilepsy
Sources: Source: *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; Licheni et al, Developmental Medicine & Child Neurology 2018*

STOKE THERAPEUTICS

STK-001 Significantly Reduces Premature Mortality in Dravet Syndrome Mice

Significant improvements in survival among Dravet syndrome mice after STK-001 administration (20µg) at postnatal day 2

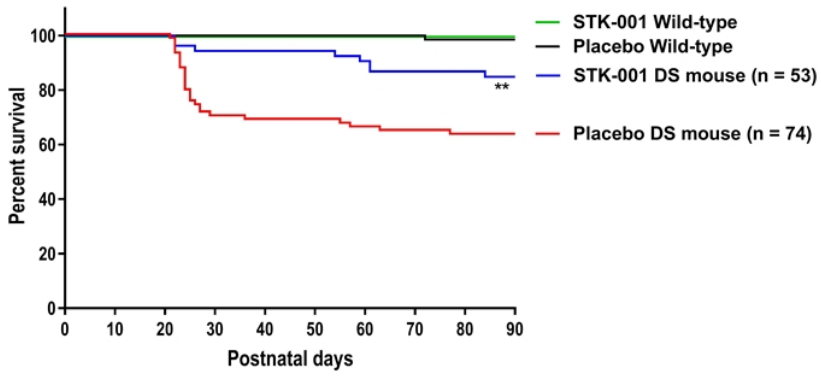


****p<0.0001

Source: Stoke data, presented at AES 2019

STK-001 Significantly Reduces Premature Mortality in Dravet Syndrome Mice

Significant improvements in survival among Dravet syndrome mice after STK-001 administration (60µg) at postnatal day 14



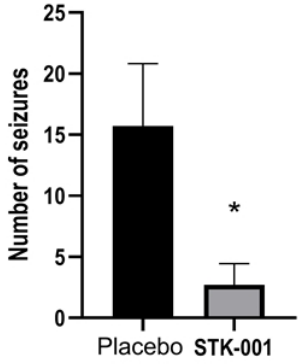
**p<0.005

Source: Stoke data, presented at AES 2019

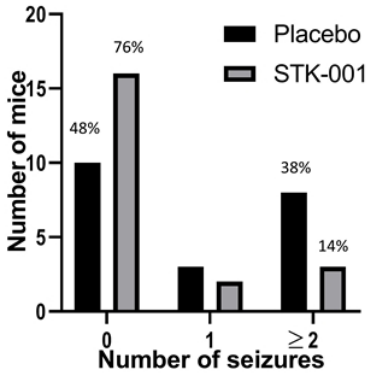
STOKE THERAPEUTICS

STK-001 Significantly Reduces Spontaneous Seizures in Dravet Syndrome Mice

80% reduction in the average number of spontaneous seizures compared to placebo (p<0.05)



76% of mice treated with STK-001 were seizure-free compared to 48% of placebo-treated mice



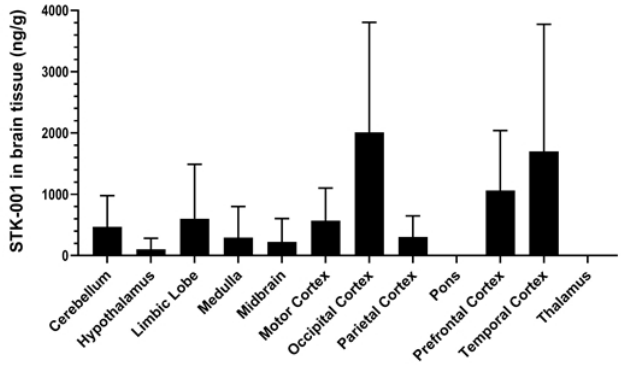
As measured between postnatal days (PND) 22 and 46 in Dravet syndrome mice after a single 20 µg injection of STK-001 at PND 2

Source: Stoke data, presented at AES 2019

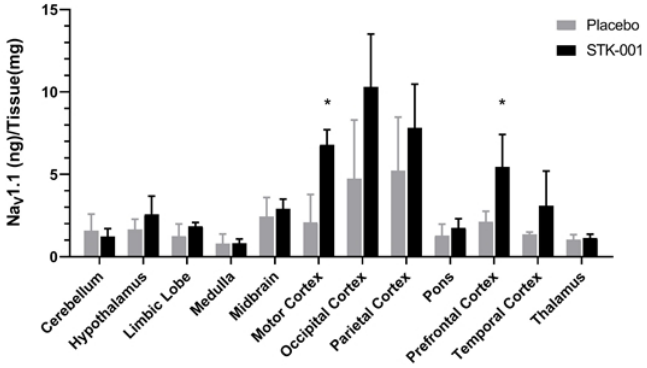
STOKE THERAPEUTICS

STK-001 Achieves Broad Distribution and Increases $Na_V1.1$ Protein Expression in NHPs (n=3)

Exposure of STK-001 observed in all brain regions except pons and thalamus



$Na_V1.1$ protein levels increased up to 3-fold



Source: Stoke data, presented at AES 2019

STOKE THERAPEUTICS

Preclinical Studies Show STK-001 Well-Tolerated at a Pharmacologically Active Dose in NHPs

Key safety measures

No complement activation	✓
No decrease in platelet counts	✓
No change in renal or hepatic function	✓
No clinical signs or symptoms over 28-day period after administration	✓
Normal histopathology in brain, liver and kidney	✓

Source: Stoke data, presented at AES 2019

STOKE THERAPEUTICS

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

Preclinical data support the use of STK-001 in Dravet syndrome:

- ✓ Significant reduction in mortality and seizure frequency in Dravet syndrome mouse model
- ✓ Broad distribution to the brains of non-human primates with intrathecal delivery
- ✓ Ability to dose titrate with wide therapeutic window
- ✓ Effects persisting for at least 14 weeks in Dravet syndrome mice following a single dose
- ✓ Well-tolerated at pharmacologically-active dose levels in non-human primates
- ✓ Selective target engagement may limit potential off-target effects

STOKE THERAPEUTICS

Several Factors De-Risk STK-001 Clinical Development for Dravet Syndrome

Defined patient population:

Genetic testing routine in epilepsy diagnostic workups

Validated chemistry and delivery to CNS:

Years of experience support use of both

Established regulatory pathway:

Existing medicines are approved for Dravet syndrome

No internal manufacturing build-out required:

Contract manufacturing is established and highly scalable

STOKE THERAPEUTICS

BUTTERFLY Observational Study Ongoing

BUTTERFLY
An observational study of Dravet syndrome patients

- Two-year observational study of children and adolescents ages 2-18
- Designed to evaluate seizure frequency and non-seizure comorbidities associated with Dravet syndrome, including:
  - Intellectual disabilities
  - Developmental disabilities
  - Motor impairment
  - Speech impairment
  - Behavioral problems
  - Sleep abnormalities

STOKE THERAPEUTICS

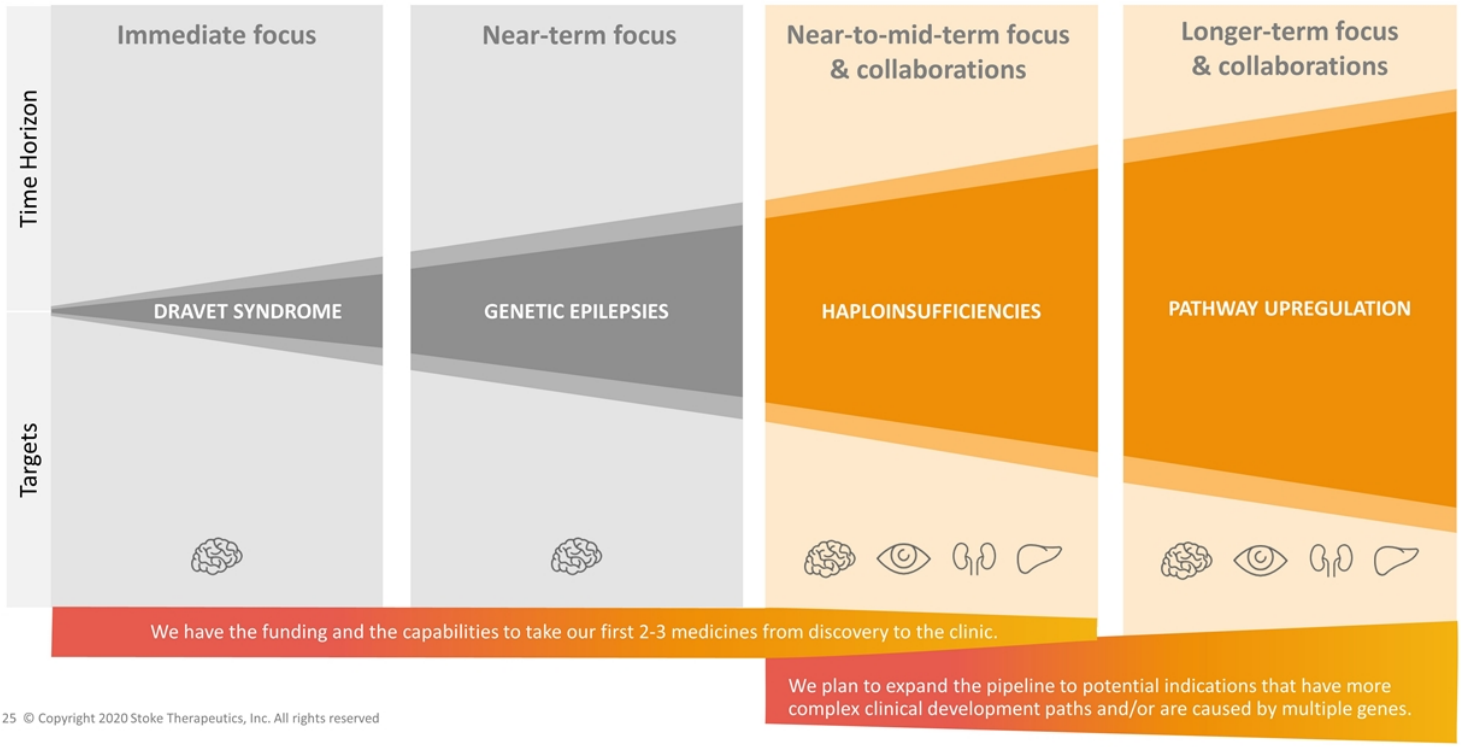
On-Track to Enroll First Patient in MONARCH Phase 1/2a Trial in 2Q/3Q 2020

- Pre-IND meeting conducted and IND on track
- GLP single-dose toxicology studies completed
- Overview of study design:
  - Analogous trial design and endpoints to recently approved antiepileptic drugs for Dravet syndrome
    - Open label
    - Plan to enroll ~40 patients aged 2-18 at ~20 sites in the U.S.
    - For each dose level, sentinel group ages 13-18, followed by group ages 2-12
    - Primary endpoint: safety and tolerability of a single-ascending dose
  - Secondary endpoints: change in seizure frequency over 12-weeks, cognitive function, quality of life
- Initial data expected in 2021

monarch
A DRAVET SYNDROME STUDY

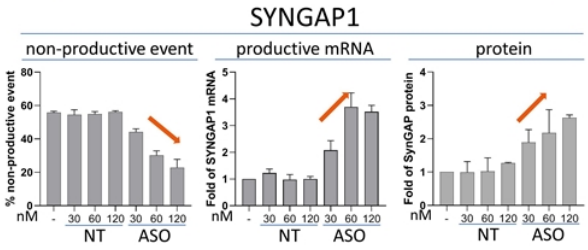
STOKE THERAPEUTICS

Expanding the Pipeline Using Stoke's Proprietary Bioinformatics and TANGO

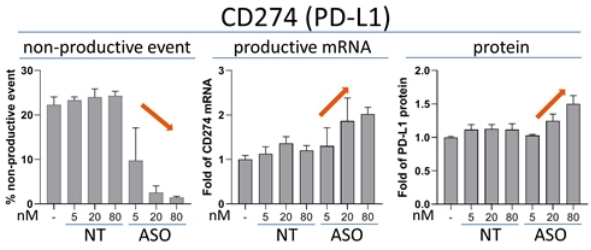


TANGO is Applicable to a Broad Range of Targets

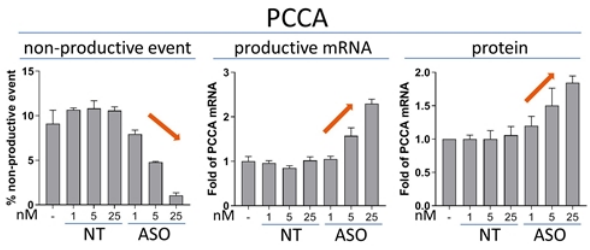
Genetic epilepsy – haploinsufficiency



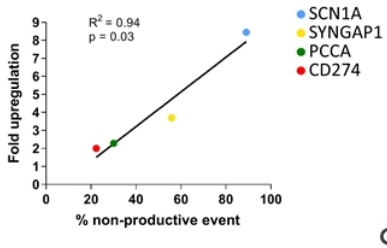
Pathway target – wild type



Liver target – autosomal recessive



Correlation between event abundance (+CHX) & upregulation



NT: non-targeting ASO control, all experiments n = 3, *in vitro*

Business Development Vision – Sector Expert & Collaborator of Choice

Numerous validated targets in therapeutic areas outside of Stoke's internal scope: metabolic, renal, immunology, cancer, hematology, neuromuscular, as well as partner-proprietary targets

Explore alternative drug delivery approaches to expand into tissues poorly accessed by ASOs and provide improved product profiles

Strategic collaborations in above areas will bolster our pipeline and more fully exploit the potential of our TANGO platform and proprietary bioinformatics

With our expertise we believe we can **drive partnered programs rapidly to clinical proof of concept**

STOKE THERAPEUTICS

Rapidly Scaling Stoke to Support Growth as a Clinical-Stage Company

2018

Stoke is Launched

2019

Stoke is Poised to Enter the Clinic

2020

Stoke Becomes a Clinical-Stage Company

- ❑ Early 2020: File IND with FDA
- ❑ 2Q/3Q 2020: Enroll first patient in MONARCH
- ❑ Mid-2020: Nominate new candidate for preclinical development in an additional genetic disease
- ❑ Continuously evaluate potential collaborations to expand the pipeline
- ❑ Build the organization and capabilities to scale as a clinical-stage company

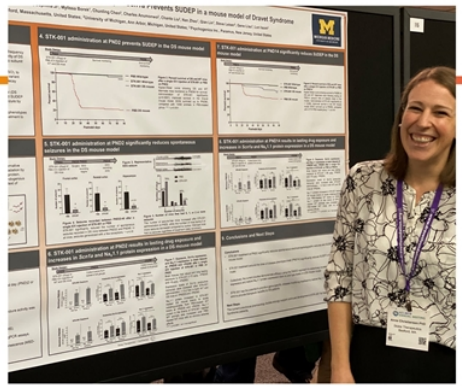
STOKE THERAPEUTICS

Cash Expected to be Sufficient to Fund Operations into 2023

Cash, Cash Equivalents and Restricted Cash *as of 09/30/2019*	**$233.2 million**
Common Shares Outstanding *as of 09/30/2019*	**32,724,153**

Raised $151.9 million in net proceeds in June 2019 initial public offering

STOKE THERAPEUTICS



The Commitment that Drives Stoke

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