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 8, 2024

Stoke Therapeutics, Inc.
(Exact name of Registrant as Specified in Its Charter)

Delaware	**001-38938**	**47-1144582**
(State or Other Jurisdiction of Incorporation)	**(Commission File Number)**	**(IRS 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

(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.

On January 8, 2024, Stoke Therapeutics, Inc., a Delaware corporation (the "Company"), posted an updated corporate presentation with additional information to its website, in advance of making a formal presentation of such information (the "Presentation") at the J.P. Morgan Healthcare Conference on January 10, 2024. The Company is furnishing a copy of the Presentation, a full copy of which is attached hereto as Exhibit 99.1.

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 8.01 Other Events.

Additionally, on January 8, 2024, the Company announced updated anticipated timing of certain milestones, including the following:

STK-001: Dravet Syndrome

- In the first quarter of 2024, the Company plans to report additional clinical and modeling data from 81 patients treated in the Phase 1/2a studies of STK-001 (MONARCH and ADMIRAL) and the two ongoing open-label extension studies (OLE) (SWALLOWTAIL and LONGWING), including:
    - Safety, pharmacokinetic modeling, and cerebrospinal fluid results;
    - Seizure frequency data from approximately 20 patients who received 1, 2, or 3 initial doses of 70mg of STK-001 and were followed for six months;
    - The effects of repeat doses of STK-001 (30mg, 45mg) on seizure frequency and cognition and behavior from patients treated in the SWALLOWTAIL and LONGWING OLE studies.
- Pending the results of the Q1 data readout, the Company plans to proceed with Phase 3 preparation activities, including discussions with global regulatory agencies, availability of chronic toxicology data, preparation of the investigator brochure, submission of a final protocol to regulatory agencies and institutional review boards.

STK-002: Autosomal Dominant Optic Atrophy

- Phase 1 study (OSPREY) of STK-002 is expected to start in the UK in 2024.

Item 9.01 Financial Statements and Exhibits.

(d) Exhibits

Exhibit Number	Description
99.1	Presentation, dated as of January 2024.
104	Cover Page Interactive Data File (the cover page XBRL tags are embedded within the inline XBRL document).

Forward-Looking Statements

This Current Report on Form 8-K contains forward-looking statements within the meaning of the U.S. Private Securities Litigation Reform Act of 1995 and other federal securities laws. Any statements contained herein that do not describe historical facts, including, but not limited to the ability of STK-001 to treat the underlying causes of Dravet syndrome and reduce seizures or show improvements in behavior or cognition at the indicated dosing levels or at all; the timing and expected progress of clinical trials, data readouts and presentations for STK-001 and STK-002; the timing of regulatory interactions or the outcome thereof; and the Company's cash runway. Statements including words such as "anticipate," "plan," "will," "continue," "expect," 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 prove incorrect or do not fully materialize, could cause our results to differ materially from those expressed or implied by such forward-looking statements, including, but not limited to, risks and uncertainties related to: the Company's ability to advance, obtain regulatory approval of, and ultimately commercialize its product candidates; the timing of data readouts and interim and final results of preclinical and clinical trials; positive results in a clinical trial may not be replicated in subsequent trials or successes in early stage clinical trials may not be predictive of results in later stage trials; preliminary interim data readouts of ongoing trials may show results that change when such trials are completed; the Company's ability to fund development activities and achieve development goals into 2025; the Company's ability to protect its intellectual property; the direct or indirect impact of global business, political and macroeconomic conditions, including inflation, interest rate volatility, cybersecurity events, uncertainty with respect to the federal budget, instability in the global banking system and volatile market conditions, and global events, including public health crises, and ongoing geopolitical conflicts, such as the conflicts in Ukraine and the Middle East; and other risks and uncertainties described under the heading "Risk Factors" in the Company's Annual Report on Form 10-K for the year ended December 31, 2022, its quarterly reports on Form 10-Q, and the other documents the Company files from time to time with the Securities and Exchange Commission. These forward-looking statements speak only as of the date hereof, and the Company undertakes no obligation to revise or update any forward-looking statements to reflect events or circumstances after the date hereof.

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 hereunto duly authorized.

STOKE THERAPEUTICS, INC.

Date: January 8, 2024

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

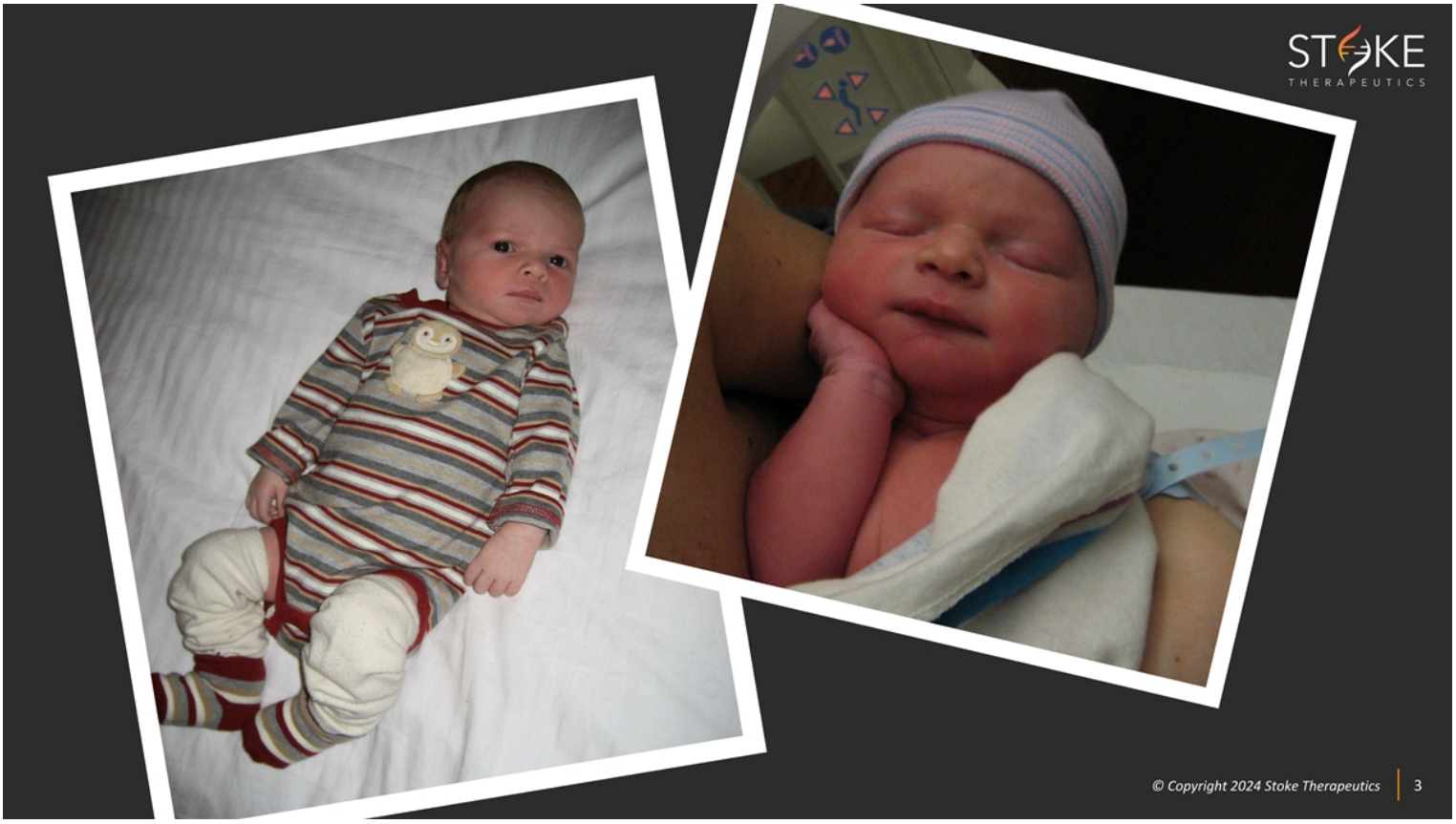
42nd Annual J.P. Morgan Healthcare Conference
January 10, 2024

Disclaimer

This presentation has been prepared by Stoke Therapeutics, Inc. ("Stoke" or "us") 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 STK-001 to treat the underlying causes of Dravet syndrome and reduce seizures or show improvements in behavior or cognition at the indicated dosing levels or at all; the timing and expected progress of clinical trials, data readouts and presentations for STK-001 and STK-002; the timing of regulatory interactions or the outcomes thereof; our future operating results, financial position and cash runway; and our expectations, plans, aspirations and goals, including those related to the goals of our collaboration with Acadia. Statements including words such as "anticipate," "plan," "will," "continue," "expect," 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 prove incorrect or do not fully materialize, could cause our results to differ materially from those expressed or implied by such forward-looking statements, including, but not limited to, risks and uncertainties related to: Stoke's ability to advance, obtain regulatory approval of, and ultimately commercialize its produce candidates; the timing of data readouts and interim and final results of preclinical and clinical trials; positive results in a clinical trial may not be replicated in subsequent trials or successes in early stage clinical trials may not be predictive of results in later stage trials; preliminary interim data readouts of ongoing trials may show results that change when such trials are completed; Stoke's ability to fund development activities and achieve development goals into 2025; Stoke's ability to protect its intellectual property; global business, political and macroeconomic conditions, including inflation, interest rate volatility, cybersecurity events, uncertainty with respect to the federal budget, instability in the global banking system and volatile market conditions, and global events, including public health crises, and ongoing geopolitical conflicts, such as the conflicts in Ukraine and the Middle East; and other risks and uncertainties described under the heading "Risk Factors" in Stoke's Annual Report on Form 10-K for the year ended December 31, 2022, its quarterly reports on Form 10-Q and the other documentation Stoke files from time to time with the Securities and Exchange Commission. These forward-looking statements speak only as of the date of this presentation, and we undertake no obligation to revise or update any forward-looking statements to reflect events or circumstances after the date hereof.

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.



1

out of

16,000

babies are born
with Dravet syndrome

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

~35,000

people affected in:

United States
Canada
Japan
Germany
France
United Kingdom

Sources: *2018 Health Advances Report*

50% of Epilepsies are Unknown or Idiopathic; Majority of These are Now Thought to be Genetic

Epilepsy affects approximately 1.2 % of the US population

Causes:

- Trauma
- Tumor
- Stroke
- Infection
- Developmental abnormalities of brain

However, 50% of epilepsies are due to genetic mutations (previously known as cryptogenic)

~85%

of Dravet cases caused by a **HAPLOINSUFFICIENCY** of the *SCN1A* gene

Resulting in

50%

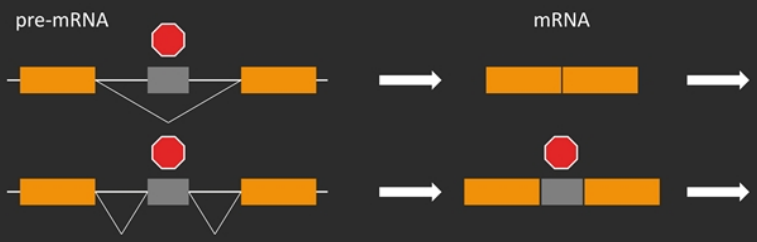
$Na_V1.1$ protein expression

Sources: The Epilepsy Foundation; Centers for Disease Control and Prevention; Nabbout et al., Orphanet Journal of Rare Diseases, 2013; Dravet Syndrome Foundation Voice of the Patient Report

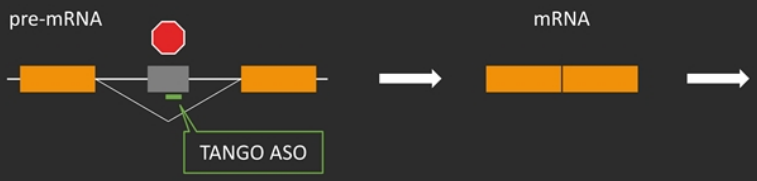
Our Goal: Upregulate Protein Expression to Treat the Underlying Cause of Severe Genetic Diseases

Mechanism of action for TANGO

Non-productive exons control protein levels by alternative splicing



TANGO ASOs skip out non-productive exons



TANGO: Targeted Augmentation of Nuclear Gene Output
PTC: premature termination codon
NMD: nonsense-mediated mRNA decay

The Effects of Dravet Go Beyond "Just Seizures"

STOKE THERAPEUTICS

~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

Time Loss

Intellectual Disability & Developmental Delays

Growth Defects

Side Effects

Disruptions of Autonomic Nervous System

Direct Medical Costs

Burden on Caregiver

Movement & Balance

Home Modifications

Burden on Siblings

ER Visits

Career Sacrifices

Mood Disorders

Language & Speech Disturbances

Sleep Abnormalities

Multiple Daily Medicines

Forced Retirement

Keto Diet

[1] *Sudden Unexpected Death in Epilepsy.*

Sources: Lagae et al., Developmental Medicine & Child Neurology, 2017; 2018 Health Advances Report; Dravet Syndrome Foundation Voice of the Patient Report

STK-001 is on Track to be the First Disease-Modifying Treatment for Dravet Syndrome

Multiple medicines available for

Seizure management

Available medicines used to control seizures:

- Acetazolamide
- Benzodiazepines
- Brivaracetam
- Cannabidiol
- Carbamazepine
- Clobazam
- Ethosuximide
- Felbamate
- Fenfluramine
- Lamotrigine
- Levetiracetam
- Mesuximide
- Oxcarbazepine
- Phenytoin
- Rufinamide
- Stiripentol
- Topiramate
- Valproate products
- Zonisamide

Despite these treatments, seizures are not adequately controlled in 90% of patients with Dravet syndrome

No medicines available for

Syndrome management

STK-001

The first potential disease-modifying approach to address the genetic cause of Dravet syndrome

Data Support the Potential for Disease Modification with STK-001

BUTTERFLY Natural History Data

(patients 2-18 taking standard anti-seizure medicines)

- ✕ No meaningful improvement in seizure frequency

- ✕ Widening gap in cognition and behavior:
  - Expressive communication
  - Receptive communication
  - Gross motor skills

Data from Phase 1/2a and OLE Clinical Studies

- ✓ Substantial and sustained reductions in seizures with initial treatment (70mg)
  - Median reductions observed among all patients at 3 and at 6 months
  - Sustain reductions with ongoing treatment

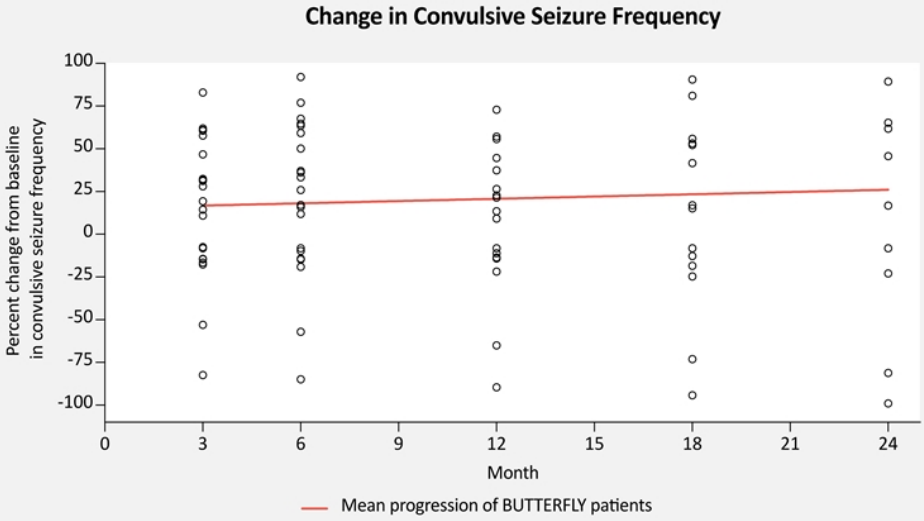
- ✓ Substantial improvements in measures of cognition and behavior:
  - Expressive communication
  - Receptive communication
  - Gross motor skills

Safety
Generally well-tolerated in studies to date

PK data
Higher drug exposure in brain leads to greater reductions in seizure frequency

No Improvement in Convulsive Seizure Frequency Despite Treatment with Standard Anti-Seizure Medicines Over 2 Years

BUTTERFLY natural history study of 2-18 years old patients with Dravet syndrome

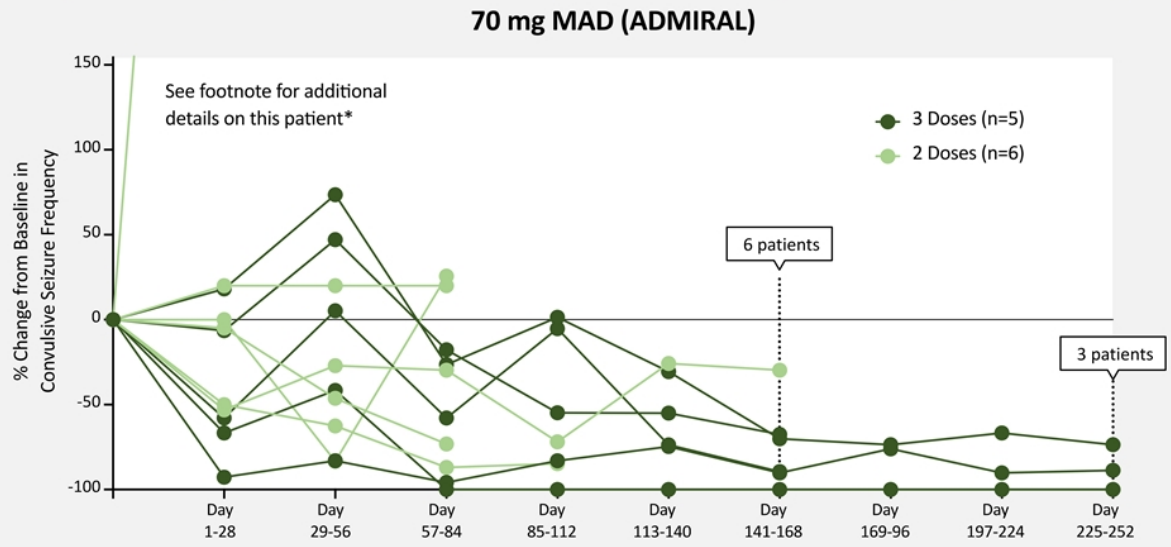


Patients were treated with the best available anti-seizure medicines	
Median baseline convulsive seizure frequency per 28 days (95% CI), n=26	
10.0 (5.50, 15.5)	
Most common ongoing anti-seizure medicines, n (%)	
Clobazam	25 (69.4%)
Fenfluramine	16 (44.4%)
Stiripentol	14 (38.9%)
Valproic Acid	14 (38.9%)
Cannabidiol	12 (33.3%)
Levetiracetam	8 (22.2%)

Source: 24-Month Analysis of BUTTERFLY: A Prospective, Observational Study to Investigate Cognition and Other Non-seizure Comorbidities in Children and Adolescents with Dravet Syndrome (DS), AES 2023.

Substantial Reductions in Seizure Frequency Observed in Patients Treated with 2 or 3 Initial Doses of STK-001 (70mg)

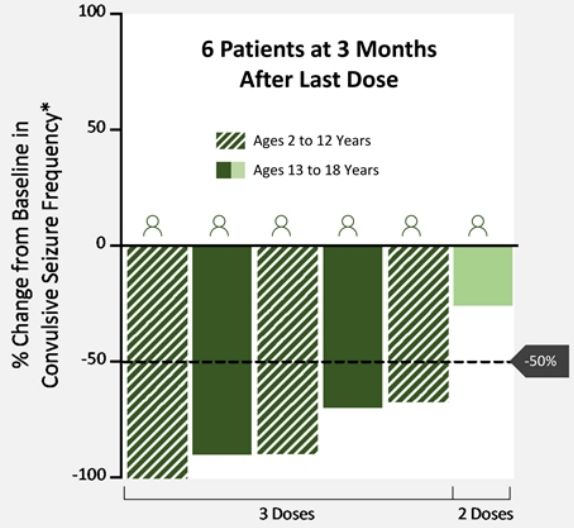
Data as of July 2023. All patients have now completed the study. End of study data anticipated 1Q 2024.

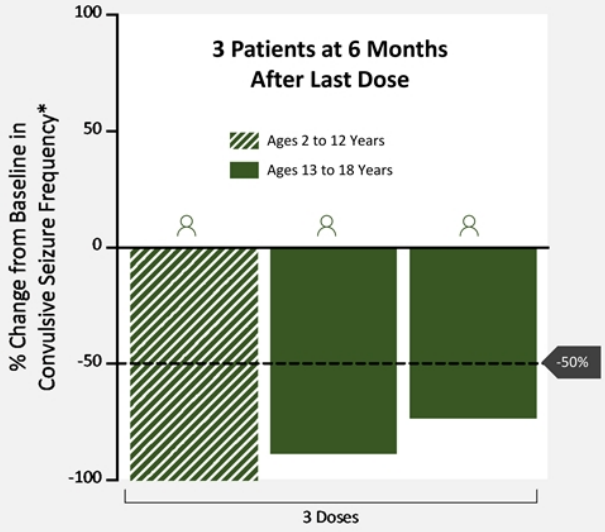


*D1-D28: +825%, D29-D56: +626%, D57-D84: +1125%, D85-D112: +717%

Source: MONARCH and ADMIRAL: Phase 1/2a Studies in US and UK Investigating Safety and Drug Exposure of STK-001, an Antisense Oligonucleotide (ASO), in Children and Adolescents with Dravet Syndrome (DS), AES 2023.

Median Reductions in Convulsive Seizure Frequency Observed Among All Patients with 2 or 3 Doses of 70mg at 3 and at 6 Months



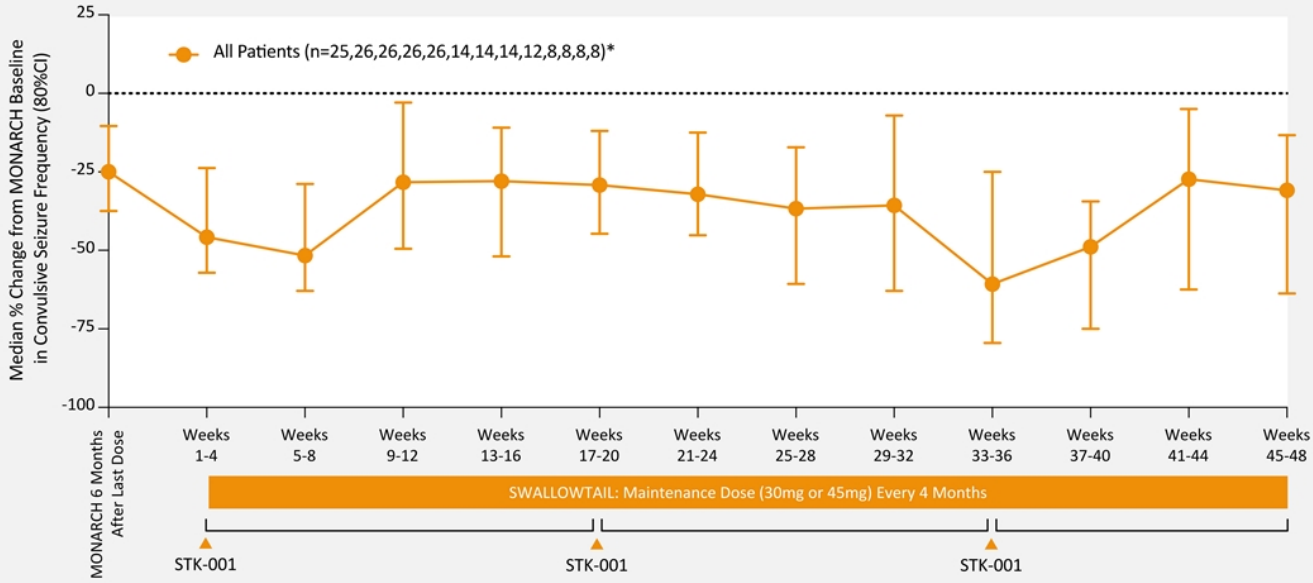


*28-day interval prior to 3 months or 6 months After Last Dose for all patients. 1 patient in 70 mg cohort received Dose 3 late; therefore, interval does not extend fully to 3 and 6 months After Last Dose for this patient. Data cutoff dates: MONARCH 13APR2023; ADMIRAL 12APR2023 and 21JUN2023

Source: MONARCH and ADMIRAL: Phase 1/2a Studies in US and UK Investigating Safety and Drug Exposure of STK-001, an Antisense Oligonucleotide (ASO), in Children and Adolescents with Dravet Syndrome (DS), AES 2023.

OLE Data: Sustained Reductions in Convulsive Seizure Frequency

Effects observed with ongoing treatment with STK-001 at 30mg, 45mg

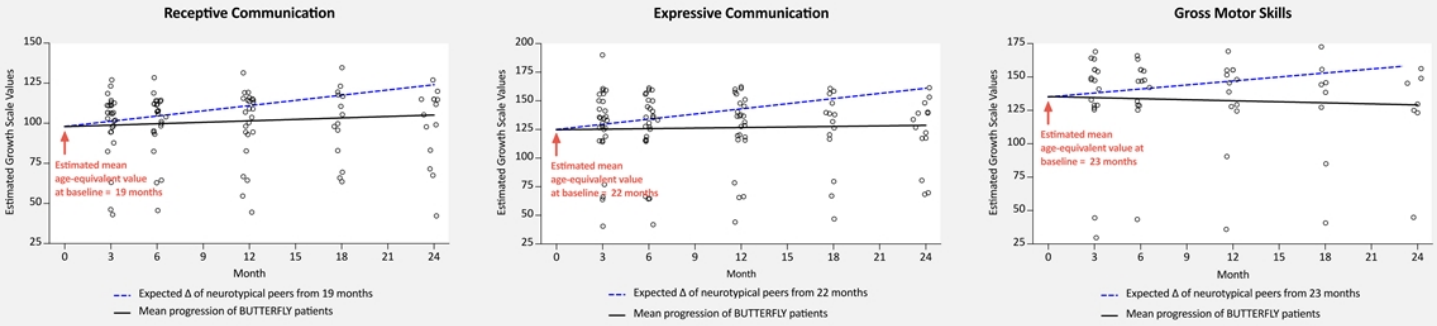


No exclusion for AED modification in MONARCH or SWALLOWTAIL. Data cutoff dates: MONARCH 13APR2023; SWALLOWTAIL 24MAR2023

Source: SWALLOWTAIL and LONGWING: Open-Label Extension (OLE) Studies for Children and Adolescents with Dravet Syndrome (DS) who Previously Participated in a Study of Antisense Oligonucleotide (ASO) STK-001, AES 2023.

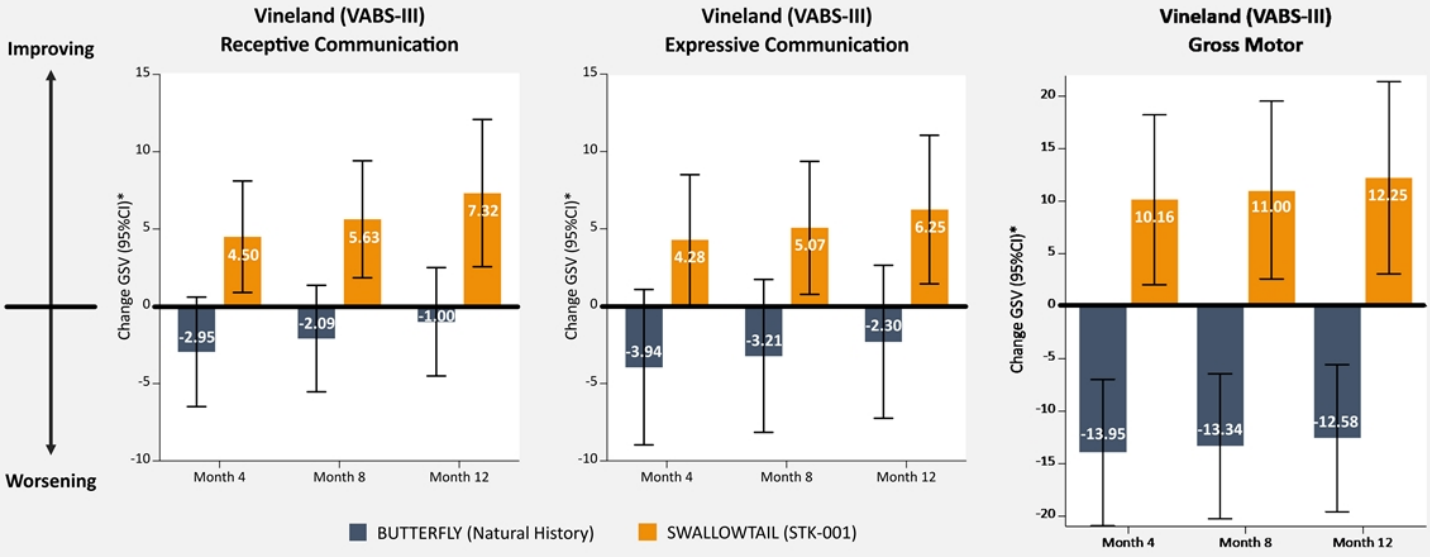
Natural History Shows Widening Gap in Cognition and Behavior Compared to Neurotypical Peers

Dravet patients were taking standard anti-seizure medicines throughout the 2-years of study



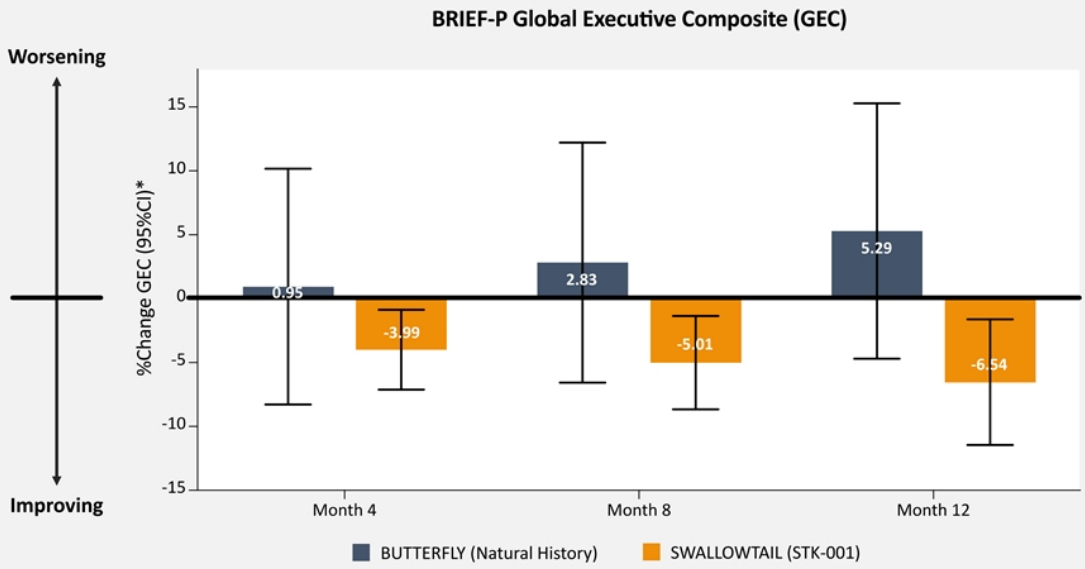
Source: 24-Month Analysis of BUTTERFLY: A Prospective, Observational Study to Investigate Cognition and Other Non-seizure Comorbidities in Children and Adolescents with Dravet Syndrome (DS), AES 2023.

OLE Data (30mg, 45mg): Substantial Improvements in Communication and Gross Motor Skills with Ongoing Treatment



**Mixed model repeated measures with AR(1) covariance structure. Baseline covariates in BUTTERFLY matched to SWALLOWTAIL. Analysis includes patients who received 30 or 45 mg for all doses in SWALLOWTAIL; BUTTERFLY sample size: n=36 at screen, n=27 at Month 12; SWALLOWTAIL sample size: n=24 at screen, n=9 at Week 48 and n=5 at Week 64. GSV = Growth Scale Value.*
Source: SWALLOWTAIL and LONGWING: Open-Label Extension (OLE) Studies for Children and Adolescents with Dravet Syndrome (DS) who Previously Participated in a Study of Antisense Oligonucleotide (ASO) STK-001, AES 2023.

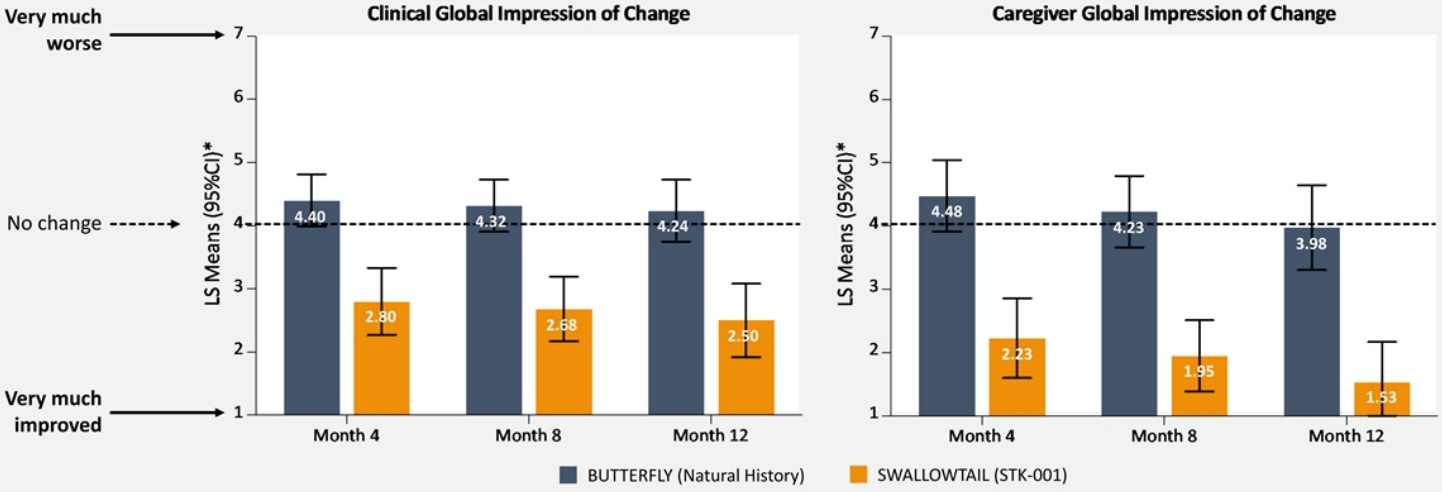
OLE Data (30mg, 45mg): Substantial Improvements in Executive Function with Ongoing Treatment



*Mixed model repeated measures with AR(1) covariance structure. Baseline covariates in BUTTERFLY matched to SWALLOWTAIL. Analysis includes all patients who received 30 or 45 mg for all doses in SWALLOWTAIL; BUTTERFLY sample size: n=36 at screen, n=30 at Month 12; SWALLOWTAIL sample size: n=25 at screen, n=9 at Week 48 and n=5 at Week 64. BRIEF-P measures executive function in children, such as the ability to organize thoughts and have working memory. Source: SWALLOWTAIL and LONGWING: Open-Label Extension (OLE) Studies for Children and Adolescents with Dravet Syndrome (DS) who Previously Participated in a Study of Antisense Oligonucleotide (ASO) STK-001, AES 2023.

OLE Data (30mg, 45mg): Substantial Improvements in Overall Condition Compared to BUTTERFLY Natural History Results

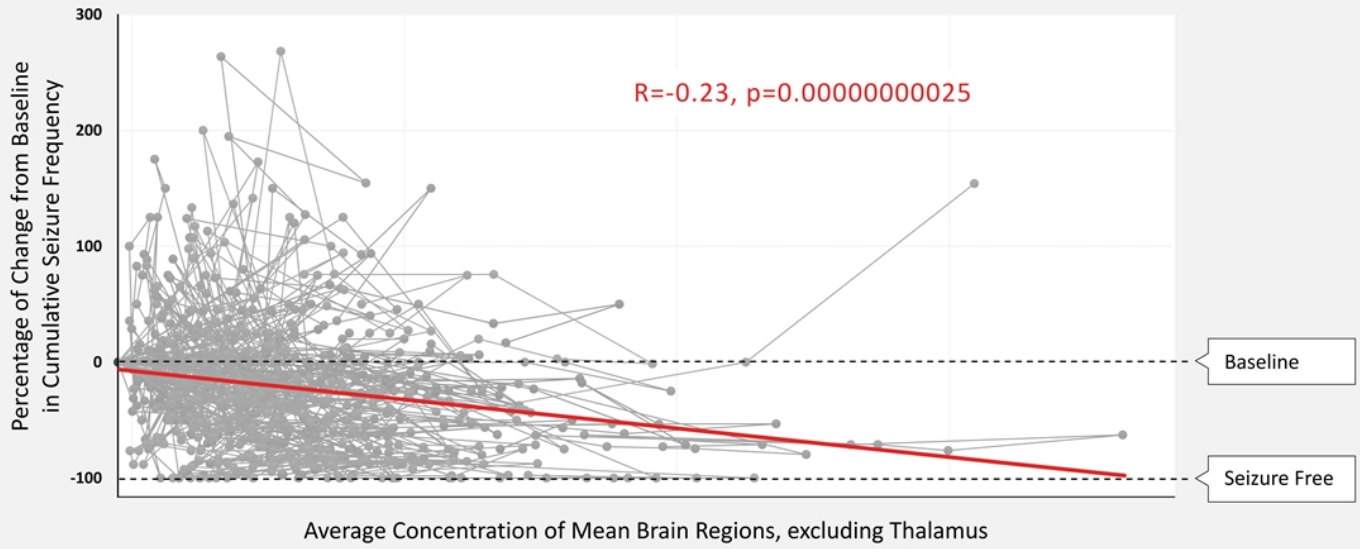
Consistent responses across caregiver and clinician ratings



Mixed model repeated measures with AR(1) covariance structure. Baseline covariates in BUTTERFLY matched to SWALLOWTAIL. Analysis includes all patients who received 30 or 45 mg for all doses in SWALLOWTAIL. For CGI-C, BUTTERFLY sample size: n=32 at Month 3, n=29 at Month 12; and for CaGI-C, BUTTERFLY sample size: n=27 at Month 3, n=24 at Month 12. For both CGI-C and CaGI-C, SWALLOWTAIL sample size: n=25 at Week 16, n=9 at Week 48 and n=5 at Week 64. CGI and CaGI in BUTTERFLY were adapted for cognition. CGI-C=Clinical Global Impression of Change and CaGI-C=Caregiver Global Impression of Change. Sources: SWALLOWTAIL and LONGWING: Open-Label Extension (OLE) Studies for Children and Adolescents with Dravet Syndrome (DS) who Previously Participated in a Study of Antisense Oligonucleotide (ASO) STK-001, AES 2023.

Higher Brain Exposure Leads to Greater Seizure Reduction

PK Modeling of exposure-seizure relationship



Source: Utilization of a Pharmacokinetic (PK) Model for STK-001 in Patients with Dravet Syndrome (DS) To Support Selection of Dosing Regimens in Clinic, AES 2023.

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

Summary of Key Clinical Data from Ongoing Studies

- ✓ Single and multiple doses of 10mg to 70mg were generally well-tolerated
- ✓ Patients treated with 2 or 3 doses of 70mg experience substantial and sustained reductions in convulsive seizures
- ✓ Reductions in seizure frequency were maintained with ongoing treatment at lower doses (30mg, 45mg)
- ✓ Improvements in assessments of cognition and behavior as measured by VABS-III* & BRIEF-P**

*Vineland Adaptive Behavior Scale (VABS-III), an assessment of adaptive behavior which refers to an individual's ability to undertake daily activities appropriate for their age group.
**Behavior Rating Inventory of Executive Function–Preschool Version, an assessment of pediatric executive function.

Q1 2024 Data Readout & Next Steps

Q1 2024
Key Anticipated Data

- ❑ Safety, pharmacokinetic (PK) modeling, and cerebrospinal fluid (CSF)
- ❑ Seizure frequency from ~20 patients who received 1, 2, or 3 initial doses of STK-001 (70mg) and were followed for six months
- ❑ Seizure frequency, cognition and behavior from patients treated in the OLE studies

Rest of Year
Phase 3 Preparations

- ❑ Global regulatory interactions
- ❑ Chronic toxicology data
- ❑ Protocol finalization/submission
- ❑ Investigator brochure
- ❑ Seek IRB approvals

2024 Summary of Priorities

Advance STK-001 for Dravet Syndrome to Pivotal

- Q1 Data Readout
- Pending data, request Phase 3 planning meetings with regulators

Advance STK-002 for ADOA

- Initiate Phase 1 study (OSPREY) in 2024

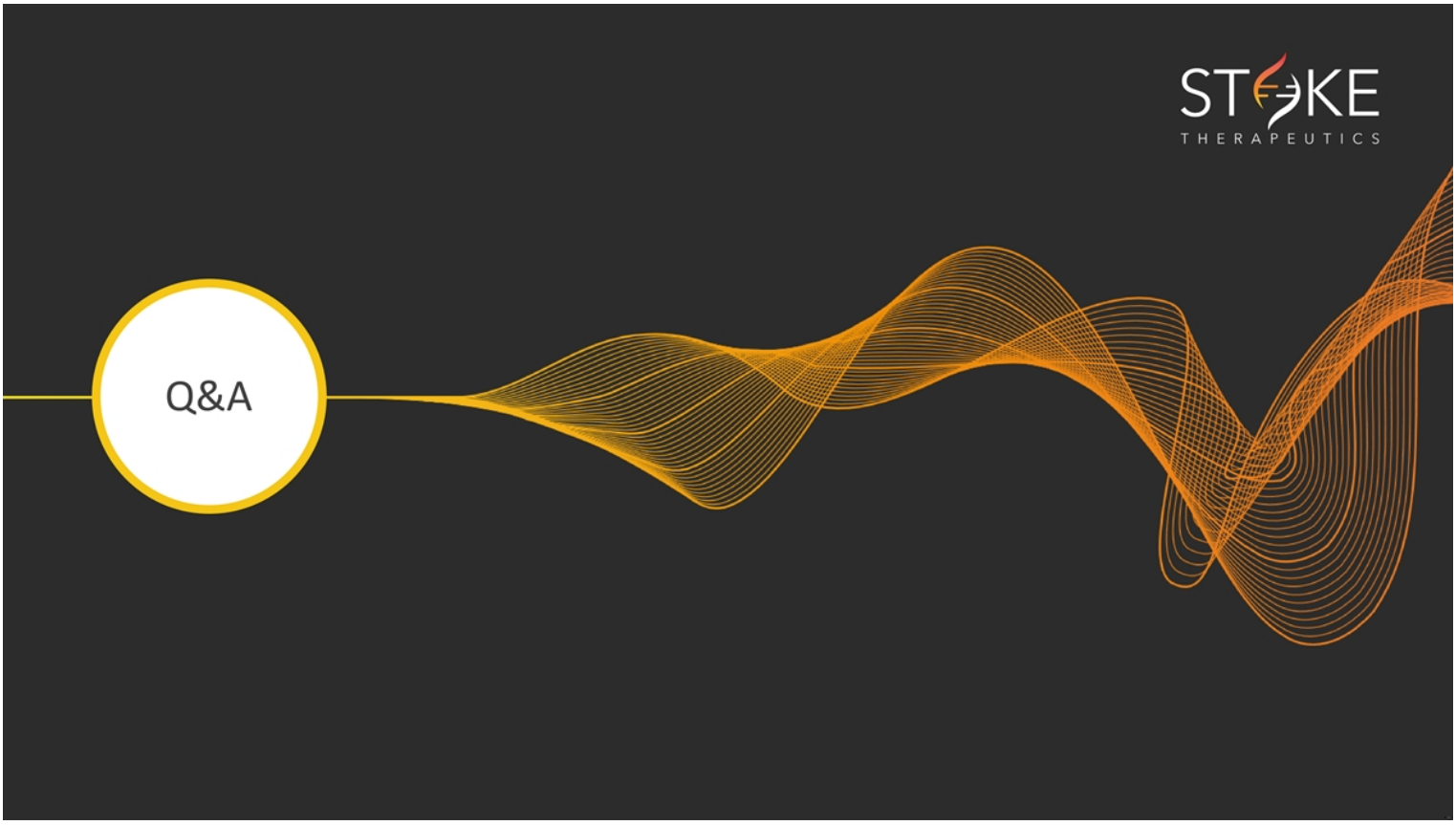
Develop & Expand Pipeline

- Execute on collaboration with Acadia to advance 3 neurodevelopmental programs including Rett syndrome and Syngap1 programs
- Expand TANGO ASOs as a first-in-class disease-modifying approach for additional genetic diseases

Current Liquidity Anticipated to Fund Operations to the End of 2025

$214.7M in Cash, Cash Equivalents, and Marketable Securities as of 9/30/23

ADOA: Autosomal dominant optic atrophy


STOKE
THERAPEUTICS
Q&A