Stoke Therapeutics Announces First Quarter 2026 Financial Results and Provides Business Updates

May 7, 2026

– New 4-year longitudinal data from the Phase 1/2a open-label extension (OLE) studies provide additional support for the disease-modifying potential of zorevunersen, an investigational medicine for the treatment of Dravet syndrome –

– Statistically significant improvements in cognition and behavior demonstrated at 1, 2, 3 and 4 years of treatment compared to OLE baseline, in addition to continued durability in seizure reductions –

– Zorevunersen generally well tolerated, with some patients treated for more than 5 years –

– Enrollment of approximately 150 patients into the Phase 3 EMPEROR study expected to complete in June 2026 to support a data readout in mid-2027; these data are anticipated to complete the rolling U.S. NDA submission planned to initiate in first quarter 2027 –

– As of March 31, 2026, the Company had $411.0 million in cash, cash equivalents and marketable securities expected to fund operations into 2028, including $80.7 million raised through selective ATM sales in first quarter 2026 –

– Webcast and conference call for analysts and investors at 4:30PM Eastern Time today –

BEDFORD, Mass.--(BUSINESS WIRE)--May 7, 2026-- Stoke Therapeutics, Inc. (Nasdaq: STOK) is a biotechnology company dedicated to restoring protein expression by harnessing the body's potential with RNA medicine and has a lead investigational medicine, zorevunersen, in development with Biogen (Nasdaq: BIIB) as a first-in-class potential disease-modifying treatment for Dravet syndrome. The Company today reported financial results for the first quarter ended March 31, 2026, and announced new 4-year longitudinal data from the ongoing Phase 1/2a open-label extension (OLE) studies that provide additional support for zorevunersen as a potential disease-modifying treatment for Dravet syndrome. Statistically significant improvements were demonstrated in cognition and behavior at 1, 2, 3 and 4 years of treatment compared to OLE baseline. Reductions in major motor seizure frequency were observed through 4 years of treatment in patients taking standard anti-seizure medicines (ASMs). Zorevunersen continues to be generally well tolerated, with some patients treated for more than 5 years in the Phase 1/2a and ongoing OLE studies.

The Company also announced an update on progress of the global Phase 3 EMPEROR study. Enrollment of approximately 150 patients in the U.S., UK and Japan is expected to complete in June 2026 to support a data readout in mid-2027. These data are anticipated to complete the rolling New Drug Application (NDA) to the U.S. Food and Drug Administration (FDA) planned to initiate in the first quarter of 2027.

"These new 4-year OLE data suggest that zorevunersen may change the course of Dravet syndrome by providing children with durable reductions in seizures and the possibility of a more neurotypical development path. Together with the Phase 1/2a results, the ongoing extension studies offer 5 years of clinical data, providing a unique opportunity to understand the long-term benefits and safety of zorevunersen," said Ian F. Smith, Chief Executive Officer and Director of Stoke Therapeutics.

Mr. Smith continued, "We look forward to results from our pivotal Phase 3 study, which is on track to complete enrollment of approximately 150 patients in June to support a data readout in mid-2027. As we continue to see this study through to completion, our focus turns increasingly to commercial preparedness and bringing zorevunersen to patients while also building our pipeline for the future. All of this is supported by our strong financial position taking us through to a potential U.S. launch in early 2028."

Program Highlights

Dravet syndrome (zorevunersen)

- **New 4-year safety and efficacy data from OLE studies:** Following treatment in the Phase 1/2a studies 93% (75/81) patients continued treatment in one of two OLE studies. As of the 4-year data cutoff, 77% (58/75) patients remained in these studies. The new 4-year data announced today showed that patients treated with zorevunersen on top of standard anti-seizure medicines continued to experience durable reductions in seizures and ongoing improvements in cognition and behavior. Zorevunersen continues to be generally well tolerated, with some patients treated for more than 5 years in the Phase 1/2a and ongoing OLE studies in which more than 850 doses have been administered. Elevated CSF protein lab values occurred in approximately 94% of patients of which 59% have been classified as a treatment-emergent adverse event. Importantly, no serious or severe clinical manifestations have been associated with CSF protein elevations. There have been no reports of hydrocephalus.
- **Pivotal Phase 3 EMPEROR study progress:**
  - **U.S., UK and Japan: New patient entry into screening is now closed**. As of May 5, 2026, approximately 130 patients had been randomized to zorevunersen or sham. The remaining patients required to achieve the planned enrollment of approximately 150 patients into this cohort are progressing through the 8-week screening period. Following successful completion of screening, these patients will be randomized to treatment with zorevunersen or a sham control administered via lumbar puncture (LP). The final patient is expected to be randomized in June 2026. These data are anticipated to be the final data required for completion of the planned rolling U.S. NDA

submission.
    - **Europe (Germany, France, Spain and Italy): 15 out of 16 sites are activated and screening is underway for at least 20 additional patients.** Enrollment is expected to complete in the third quarter of 2026.
- **Medical and scientific publications and communications continued to drive awareness of Dravet syndrome and the need for disease modification**.
    - In April, Stoke presented data at the American Academy of Neurology (AAN) Annual Meeting, the world's largest gathering of neurologists.
    - In March, *The New England Journal of Medicine (NEJM)* published zorevunersen data from the Phase 1/2a and OLE studies. An independent editorial that discussed the underlying genetic cause of Dravet syndrome and the disease-modifying potential of zorevunersen accompanied the manuscript.

Pipeline beyond zorevunersen

- In February, the first patient was dosed in the Phase 1 OSPREY study of STK-002 for the treatment of Autosomal Dominant Optic Atrophy (ADOA), the most common inherited optic nerve disorder. To date, two patients have been dosed and recruitment is ongoing at 7 sites across the UK, Germany, Denmark and Austria. Dose escalation of the first four cohorts will continue through 2026 and early 2027, pending safety and tolerability assessments.
- Lead optimization is underway to identify a clinical candidate for the treatment of SYNGAP1 in 2026. SYNGAP1 is a severe and rare genetic neurodevelopmental disease.

First Quarter 2026 Financial Results

- As of March 31, 2026, the Company had $411.0 million in cash, cash equivalents and marketable securities expected to fund operations into 2028. This includes approximately $80.7 million of proceeds from the sale of 2.6 million shares of common stock from selective use of the ATM facility (Controlled Equity Offering Sales Agreement) during the first quarter of 2026.
- Revenue recognized for the three months ended March 31, 2026, was $6.2 million, a decrease from $158.6 million for the same period in 2025. The decrease in revenue is primarily driven by the 2025 recognition of $150.8 million related to the IP license performance obligation related to the Biogen Agreement outside of the U.S., Canada and Mexico.
- Net loss for the three months ended March 31, 2026, was $50.0 million, or $0.79 per share, compared to a net income of $112.9 million, or $1.90 per diluted share, for the same period in 2025.
- Research and development expenses for the three months ended March 31, 2026, were $39.7 million, compared to $32.7 million for the same period in 2025. The increase of $7.0 million was driven by an increase in activities and personnel expenses to support the advancement of zorevunersen.
- Sales, general and administrative expenses for the three months ended March 31, 2026, increased to $20.0 million from $14.7 million for the same period in 2025. The increase of $5.3 million was driven by an increase in personnel and launch readiness expenses.

Stoke Webcast and Conference Call for Analysts and Investors

Stoke management will host a webcast and conference call for analysts and investors on Thursday, May 7, 2026, at 4:30PM Eastern Time. The webcast will be available on the Investors & News section of Stoke's website at https://investor.stoketherapeutics.com/. Research analysts who plan to join the call and participate in the Q&A session may register here to receive the dial-in details and a unique PIN. All other participants are invited to access the listen-only webcast by clicking here. A replay of the webcast will be archived and available for at least 90 days following the event.

About Dravet Syndrome

Dravet syndrome is a severe developmental and epileptic encephalopathy (DEE) characterized by recurrent seizures as well as significant cognitive and behavioral impairments. Most cases of Dravet are caused by mutations in one copy of the *SCN1A* gene, leading to insufficient levels of NaV1.1 protein in neuronal cells in the brain. Even when treated with the best available anti-seizure medicines (ASMs), up to 57 percent of patients with Dravet syndrome do not achieve ≥50 percent reduction in seizure frequency. Complications of the disease often contribute to a poor quality of life for patients and their caregivers. Developmental and cognitive impairments often include intellectual disability, developmental delays, movement and balance issues, language and speech disturbances, growth defects, sleep abnormalities, disruptions of the autonomic nervous system and mood disorders. Compared with the general epilepsy population, people living with Dravet syndrome have a higher risk of sudden unexpected death in epilepsy, or SUDEP; up to 20 percent of children and adolescents with Dravet syndrome die before adulthood due to SUDEP, prolonged seizures, seizure-related accidents or infections [1]. Dravet syndrome occurs globally and is not concentrated in a particular geographic area or ethnic group. Currently, it is estimated that up to 38,000 people are living with Dravet syndrome in the U.S. (~16,000), UK, EU-4 and Japan [2]. There are no approved disease-modifying therapies for people living with Dravet syndrome.

About Zorevunersen

Zorevunersen is an investigational antisense oligonucleotide that is designed to treat the underlying cause of Dravet syndrome by increasing functional NaV1.1 protein production in brain cells from the unaffected (wild-type) copy of the *SCN1A* gene. This highly differentiated mechanism of action aims to reduce seizure frequency beyond what has been achieved with anti-seizure medicines and to improve neurodevelopment, cognition and behavior. Zorevunersen has demonstrated the potential for disease modification and has been granted orphan drug designation by the FDA and the EMA. The FDA has also granted zorevunersen rare pediatric disease designation and Breakthrough Therapy Designation for the treatment of Dravet syndrome with a confirmed mutation not associated with gain-of-function, in the *SCN1A* gene. Stoke has a strategic collaboration with Biogen

to develop and commercialize zorevunersen for Dravet syndrome. Under the collaboration, Stoke retains exclusive rights for zorevunersen in the United States, Canada, and Mexico; Biogen receives exclusive rest of world commercialization rights. Zorevunersen is currently in clinical development, and its safety and efficacy have not been evaluated by any regulatory authority.

About the Phase 1/2a and Open-Label Extension Studies

Two Phase 1/2a open-label, multicenter studies evaluated the effects of zorevunersen in patients with highly refractory Dravet syndrome ages 2 to 18 years (N=81). Primary endpoints were the safety profile, plasma pharmacokinetics (PK) and exposure in cerebrospinal fluid (CSF) of single and multiple doses of zorevunersen. Secondary endpoints included percentage change from baseline in major motor seizure frequency, overall clinical status (a measure of patients' overall functioning) and quality of life. The ADMIRAL Phase 1/2a study included an exploratory endpoint to evaluate changes in neurodevelopmental status (cognition & behavior) as measured by Vineland Adaptive Behavior Scales, Third Edition (Vineland-3). The Phase 1/2a studies were completed in November 2023. Following treatment in the Phase 1/2a studies, eligible patients continued treatment with zorevunersen every four months in one of two OLEs. There was at least a 6-month gap between the last dose administered in the Phase 1/2a studies and the first dose administered in the OLEs. The primary endpoints are the safety profile of multiple doses of zorevunersen. Secondary endpoints include PK parameters, percentage change from baseline in major motor seizure frequency, change in overall clinical status, and change from baseline in quality of life. Exploratory endpoints include changes in neurodevelopment status as measured by Vineland-3. The OLE studies are ongoing.

About the Phase 3 EMPEROR Study

The Phase 3 EMPEROR Study (NCT06872125) is a global, double-blind, sham-controlled study evaluating the efficacy, safety and tolerability of zorevunersen in children ages 2 to <18 with Dravet syndrome with a confirmed variant in the *SCN1A* gene not associated with gain-of-function. Stoke expects to complete enrollment of approximately 150 patients in the United States, United Kingdom and Japan in June 2026, with a data readout on track for mid-2027 to support the submission of a New Drug Application (NDA) to the FDA. At least 20 additional patients are expected to enroll in Germany, Spain, France and Italy, with completion of enrollment anticipated in Q3 2026. Participants are randomized 1:1 to receive either zorevunersen via intrathecal administration or a sham comparator for a 52-week treatment period following an 8-week baseline period. Following the completion of the study treatment period, eligible participants will be offered ongoing treatment with zorevunersen as part of an OLE study. The primary endpoint of the study is percent change from baseline in major motor seizure frequency at week 28 in patients receiving zorevunersen as compared to sham. The key secondary endpoints are the durability of effect on major motor seizure frequency and improvements in behavior and cognition as measured by Vineland-3 subdomains, including expressive communication, receptive communication, interpersonal relationships, coping skills and personal skills. Additional endpoints include safety, Clinician Global Impression of Change (CGI-C), Caregiver Global Impression of Change (CaGI-C) and the Bayley Scales of Infant Development (BSID-IV). For more information, visit https://clinicaltrials.gov/study/NCT06872125.

About Autosomal Dominant Optic Atrophy (ADOA)

ADOA is the most common inherited optic nerve disorder, affecting approximately one in 30,000 people globally with a higher incidence of one in 10,000 in Denmark due to a founder effect. It is a rare disease that causes progressive and irreversible vision loss in both eyes starting in the first decade of life. Severity can vary and the rate of vision loss can be difficult to predict. Approximately half of people with ADOA fail driving standards and up to 46% are registered as legally blind. More than 400 different disease-causing *OPA1* variants have been reported in people diagnosed with ADOA. Currently there are no approved treatments for people living with ADOA.

About STK-002

STK-002 is a proprietary antisense oligonucleotide (ASO) in clinical development for the treatment of ADOA. Stoke believes that STK-002 has the potential to be the first disease-modifying therapy for people living with ADOA. An estimated 65% to 90% of ADOA cases are caused by variants in the *OPA1* gene, most of which lead to a haploinsufficiency resulting in 50% OPA1 protein expression and disease manifestation. STK-002 is designed to upregulate OPA1 protein expression by leveraging the non-mutant (wild-type) copy of the *OPA1* gene to restore OPA1 protein expression with the aim to maintain or improve vision in people with ADOA. Stoke has generated preclinical data demonstrating proof-of-mechanism and proof-of-concept for STK-002. STK-002 has been granted orphan drug designation by FDA as a potential new treatment for ADOA. A Phase 1 study (OSPREY) of STK-002 in people with ADOA is now underway.

About the Phase 1 OSPREY Study

The OSPREY study is a Phase 1, dose-escalating open-label study of children and adults ages 6 to 55 who have an established diagnosis of ADOA and have a confirmed disease-causing variant in the *OPA1* gene. The primary objectives for the study are to assess the safety and tolerability of single ascending doses of STK-002, as well as to determine the exposure in blood. Secondary objectives are to assess changes in visual function, ocular structure and quality of life after single doses of STK-002. The OSPREY study follows a standard dose escalation design with participants enrolled into sequential cohorts receiving increasing dose levels of STK-002. Dose escalation of the first four cohorts will continue through 2026 and early 2027, pending safety and tolerability assessments. Data from the OSPREY study will help to inform potential future development of STK-002. The OSPREY study is actively recruiting in the United Kingdom, Germany, Denmark and Austria. Additional European sites are expected to activate in the coming months.

For more information on the OSPREY study, please visit:

- https://www.ospreyclinicaltrial.com/
- https://www.isrctn.com/ISRCTN41725621

About Stoke Therapeutics

Stoke Therapeutics (Nasdaq: STOK), is a biotechnology company dedicated to restoring protein expression by harnessing the body's potential with RNA medicine. Using Stoke's proprietary TANGO (Targeted Augmentation of Nuclear Gene Output) approach, Stoke is developing antisense oligonucleotides (ASOs) to selectively restore naturally-occurring protein levels. Stoke's first medicine in development, zorevunersen, has demonstrated the potential for disease modification in patients with Dravet syndrome and is currently being evaluated in a Phase 3 study. Stoke's initial focus are diseases of the central nervous system and the eye that are caused by a loss of ~50% of normal protein levels (haploinsufficiency).

Proof of concept has been demonstrated in other organs, tissues, and systems, supporting broad potential for Stoke's proprietary approach. Stoke is headquartered in Bedford, Massachusetts. For more information, visit https://www.stoketherapeutics.com/.

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: the Company's quarterly results and cash runway; its future operating results and current or future financial position and liquidity; the ability of zorevunersen to treat the underlying causes of Dravet syndrome and reduce seizures or show improvements in behavior and cognition at the indicated dosing levels or at all; the design, timing and results of clinical studies, enrollment timelines, data readouts, regulatory submissions or decisions and other presentations for zorevunersen and STK-002; the timing and potential outcomes of meetings with regulators regarding the zorevunersen program; the ability of STK-002 to treat the underlying causes of Autosomal Dominant Optic Atrophy (ADOA) and maintain or improve vision; our expectations, plans, aspirations and goals, including those related to the potential of zorevunersen and our collaborations with Biogen and Acadia. Statements including words such as "anticipate," "expect," "plan," "will," or "may" 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 the Company's 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 for, and ultimately commercialize its product candidates; that if the Company's partners were to breach or terminate their collaboration with the Company, the Company would not obtain the anticipated financial or other benefits; the possibility that the Company and Biogen may not be successful in their development of zorevunersen and that, even if successful, they may be unable to successfully commercialize zorevunersen; the risk that 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; the development goals into 2028 and through potential U.S. launch in early 2028; and the 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, 2025, its quarterly reports on Form 10-Q, and the other documents it files 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.

Financial Tables Follow

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

	March 31, 2026	December 31, 2025
Assets		
Current assets:		
Cash and cash equivalents	$ 155,659	$ 84,220
Marketable securities - current	187,886	200,450
Accounts receivable	4,451	5,936
Prepaid expenses	10,115	8,736
Interest receivable	1,690	1,969
Other current assets	6,880	4,389
Total current assets	$ 366,681	$ 305,700
Marketable securities - long-term	67,481	106,260
Restricted cash - long-term	3,227	227
Operating lease right-of-use assets	2,493	3,101
Property and equipment, net	3,395	3,146
Total assets	$ 443,277	$ 418,434
Liabilities and stockholders' equity		
Current liabilities:		
Accounts payable	$ 6,839	$ 4,939
Accrued and other current liabilities	23,901	41,035
Deferred revenue - current portion	10,066	11,901
Total current liabilities	$ 40,806	$ 57,875
Deferred revenue - net of current portion	6,603	6,961
Other long term liabilities	991	1,141
Total long term liabilities	7,594	8,102
Total liabilities	$ 48,400	$ 65,977
Stockholders' equity		
Common stock, par value of $0.0001 per share; 300,000,000 shares authorized, 62,240,347 and 58,921,999 shares issued and outstanding as of March 31, 2026 and December 31, 2025, respectively	6	5
Additional paid-in capital	942,704	849,624
Accumulated other comprehensive (loss) income	(115)	543

Accumulated deficit	(547,718)	(497,715)
Total stockholders' equity	$ 394,877	$ 352,457
Total liabilities and stockholders' equity	$ 443,277	$ 418,434

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

	Three Months Ended March 31,	
	2026	**2025**
Revenue	$ 6,229	$ 158,569
Operating expenses:		
Research and development	39,673	32,676
Sales, general and administrative	19,974	14,653
Total operating expenses	59,647	47,329
(Loss) income from operations	(53,418)	111,240
Other income (expense):		
Interest income (expense), net	3,397	2,889
Other income	18	28
Total other income (expense)	3,415	2,917
(Loss) income before income taxes	$ (50,003)	$ 114,157
Provision for income taxes	—	1,278
Net (loss) income	$ (50,003)	$ 112,879
Net (loss) income per share:		
Basic	$ (0.79)	$ 1.95
Diluted	(0.79)	1.90
Weighted-average common shares outstanding:		
Basic	63,063,507	57,862,674
Diluted	63,063,507	59,398,600
Comprehensive (loss) income:		
Net (loss) income	$ (50,003)	$ 112,879
Other comprehensive (loss) gain:		
Unrealized (loss) gain on marketable securities	(658)	47
Total other comprehensive (loss) gain	$ (658)	$ 47
Comprehensive (loss) income	$ (50,661)	$ 112,926

References:

1. Symonds, J. et al. Early childhood epilepsies: epidemiology, classification, aetiology, and socio-economic determinants. *Brain.* 2021;144(9):2879-2891.
2. Based on Stoke Therapeutics' preliminary estimates, which scaled annual incidence to prevalence using country-specific live birth rates over the past 85 years and adjusted for Dravet-specific mortality. The estimate is based on incidence rates published by Wu et al., Pediatrics, 2015.

View source version on businesswire.com: https://www.businesswire.com/news/home/20260507458327/en/

Stoke Media & Investor Contacts:
Susan Willson
Vice President, Corporate Communications
swillson@stoketherapeutics.com
415-509-8202

Doug Snow
Director, Communications & Investor Relations
IR@stoketherapeutics.com
508-642-6485

Source: Stoke Therapeutics, Inc.