Stoke Therapeutics Reports Third Quarter 2025 Financial Results and Provides Business Updates

November 4, 2025

– Global Phase 3 EMPEROR study patient recruitment ongoing in the U.S., UK, and Japan with more than 20 patients randomized to zorevunersen or sham; Study on track to complete enrollment in second half of 2026 –

– As of September 30, 2025, the Company had $328.6 million in cash, cash equivalents, and marketable securities, anticipated to fund operations to mid-2028 –

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

BEDFORD, Mass.--(BUSINESS WIRE)--Nov. 4, 2025-- 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 as a first-in-class potential disease-modifying treatment for Dravet syndrome. The Company today reported financial results for the third quarter ended September 30, 2025 and provided business updates.

"Stoke's progress in 2025 has the Company on an important growth trajectory. We are creating an understanding of the devastating impacts of Dravet syndrome on people's lives and the potential of zorevunersen to improve outcomes for them through substantial seizure reductions and improvements in cognition and behavior," said Ian F. Smith, Chief Executive Officer and Director of Stoke Therapeutics. "In just three months since the initiation of our Phase 3 EMPEROR study, we have made significant progress with more than 20 patients randomized into dosing and a steady progression of more patients entering the screening period. At the same time, presentations of new clinical data at medical congresses have provided greater understanding of zorevunersen's disease-modifying potential that is highly differentiated from any currently available treatments. We look forward to meeting with the FDA under our Breakthrough Therapy Designation before year-end, at which time we will review the four years of safety and efficacy data from our clinical studies and discuss how we can work together to deliver zorevunersen to patients through expedited regulatory pathways."

Mr. Smith continued, "Our strong financial position is allowing us to invest in the business, including enhancing our internal capabilities to rapidly scale in preparation for potential commercialization, while also expanding our pipeline in the areas of ADOA and SYNGAP1 as well as broadening our research activities."

Third Quarter 2025 Business Highlights and Recent Developments

Dravet Syndrome (Zorevunersen)

- The global Phase 3 EMPEROR study is actively recruiting patients in the U.S., UK, and Japan. European sites are expected to initiate in the first half of 2026. The Company previously disclosed that more than 150 patients had been identified by investigators as potential study candidates. As of the end of October, more than 20 patients were randomized and approximately 35 additional patients entered the formal 8-week screening period that immediately precedes randomization to zorevunersen or sham.
- In October, the Company presented new two-year data from an analysis that was initially performed to understand the potential effects of the Phase 3 dosing regimen on cognition and behavior at the 54th Child Neurology Society (CNS) Annual Meeting. The results showed continuing improvements in cognition and behavior at two years that contrast with findings from a two-year natural history study in which patients with Dravet syndrome who were treated with standard of care showed minimal changes. In addition, similar improvements in overall clinical status were reported separately by clinicians and caregivers in 95% of patients treated with zorevunersen in the ongoing open-label extension (OLE) studies (n=19). *(press release link)*
- In September, the Company presented three-year safety and efficacy data from patients who were treated in the ongoing OLE studies following initial treatment in the Phase 1/2a studies at the 36th International Epilepsy Congress (IEC). Zorevunersen was generally well tolerated across studies to date and no new safety findings have emerged. Efficacy data showed durable reductions in major motor seizure frequency on top of standard-of-care anti-seizure medicines and continuing improvements in cognition and behavior. *(press release link)*
- The Company plans to present new data from the zorevunersen clinical development program at the American Epilepsy Society (AES) 2025 Annual Meeting, December 5–9, in Atlanta, Georgia.
- The Company is scheduled to meet with the FDA before year-end 2025 to review four years of safety and efficacy data from clinical studies of zorevunersen in patients with Dravet syndrome and to discuss how the Company and Agency can work together under our Breakthrough Therapy Designation to deliver zorevunersen to patients through potential expedited regulatory pathways.

Pipeline and Corporate

- Today, the Company announced that patient recruitment into the Phase 1 OSPREY study of STK-002 is underway in the UK. STK-002 is a potential disease-modifying medicine for the treatment of Autosomal Dominant Optic Atrophy (ADOA), the most common inherited optic nerve disorder. The OSPREY study has also been authorized by the European Medicines Authority and European sites are expected to activate in early 2026.
- In October, the Company presented 24-month data from the FALCON natural history study at the 2025 American Academy of Ophthalmology (AAO) Annual Meeting. Data provide insights into disease etiology, progression and clinical assessments that are helping to inform clinical development of STK-002. (*press release link*)
- 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.
- In October, the Company's Board of Directors appointed Ian F. Smith as Chief Executive Officer after serving as Interim CEO since March 2025 and as a Director and advisor to the Company since 2023. Mr. Smith will continue to serve as a Director on the Company's Board of Directors. Arthur Tzianabos, Ph.D., resumed his role as Chairman after serving as Executive Chairman during the CEO search. (*press release link*)

Third Quarter 2025 Financial Results

- As of September 30, 2025, the Company had $328.6 million in cash, cash equivalents, and marketable securities, anticipated to fund operations to mid-2028.
- Since September 30, 2025, the Company sold approximately 1.8 million shares of its common stock and received $48.7 million after deducting commissions based on the Controlled Equity Offering Sales Agreement.
- Revenue recognized for Q3 2025 was $10.6 million, an increase from $4.9 million in Q3 2024. The increase of $5.7 million is driven by contractual obligations under agreements with Acadia and Biogen. There was an increase of $6.7 million from global development activities related to Biogen with an offset of $1.0 million related to the Acadia agreement.
- Net loss for the three months ended September 30, 2025, was $38.3 million, or $0.65 per share, compared to a net loss of $26.4 million, or $0.47 per share, for the same period 2024.
- Research and development expenses for the three months ended September 30, 2025, increased to $37.7 million from $22.2 million for the same period 2024. The increase of $15.5 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 September 30, 2025, increased to $16.0 million from $12.7 million for the same period 2024. The increase of $3.3 million was driven by an increase in personnel and launch readiness expenses.

Year-to-Date 2025 Financial Results

- Revenue recognized for the nine months ending September 30, 2025, was $183.0 million, an increase from $13.9 million for the same period 2024. The increase of $169.1 million was primarily driven by $150.8 million related to the IP license performance obligation and $11.5 million for global development activities as part of the Biogen Agreement and $6.8 million related to the Acadia Agreement.
- Net income for the nine months ended September 30, 2025, was $51.0 million, or $0.85 per diluted share, compared to a net loss of $78.5 million, or $1.48 per share, for the same period in 2024.
- Research and development expenses for the nine months ended September 30, 2025, increased to $96.2 million from $65.7 million for the same period 2024. The increase of $30.5 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 September 30, 2025, increased to $45.9 million from $36.0 million for the same period 2024. The increase of $9.9 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 Tuesday, November 4, 2025, 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 severe, 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. More than 90 percent of patients continue to experience seizures despite treatment with the best available anti-seizure medicines. 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. 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.[1]

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 non-mutated (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.

About the EMPEROR Study

The EMPEROR Phase 3 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. Study 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. An open-label extension treatment period will allow all patients the opportunity to receive treatment with zorevunersen following the 52-week treatment period. 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), EuroQol Visual Analog Scale (EQ-VAS) and the Bayley Scales of Infant Development (BSID-IV). EMPEROR has initiated in the United States, United Kingdom, Japan and is planned for Europe. For more information on the EMPEROR study, please visit https://www.emperorstudy.com/ and https://clinicaltrials.gov/study/NCT06872125.

About Autosomal Dominant Optic Atrophy (ADOA)

ADOA is the most common inherited optic nerve disorder. 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. Roughly half of people with ADOA fail driving standards and up to 46% are registered as legally blind. More than 400 different *OPA1* variants have been reported in people diagnosed with ADOA. ADOA affects approximately one in 30,000 people globally with a higher incidence in Denmark of one in 10,000 due to a founder effect. Currently there is no approved treatment 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 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 patients 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 the FDA as a potential new treatment for ADOA. A Phase 1 study (OSPREY) of STK-002 in people with ADOA is now underway.

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, data readouts, regulatory 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 result 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 to mid-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, 2024, 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.

Reference:

1. 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.

Financial Tables Follow

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

	September 30, 2025	December 31, 2024
Assets		
Current assets:		
Cash and cash equivalents	$ 83,394	$ 127,983
Marketable securities - current	164,916	88,916
Accounts receivable	9,248	1,668
Prepaid expenses	7,747	11,117
Restricted cash - current	—	75
Interest receivable	1,589	700
Other current assets	5,543	2,297
Total current assets	$ 272,437	$ 232,756
Marketable securities - long-term	80,315	29,824
Restricted cash - long-term	721	721
Operating lease right-of-use assets	3,708	4,345
Property and equipment, net	3,083	3,909
Total assets	$ 360,264	$ 271,555
Liabilities and stockholders' equity		
Current liabilities:		
Accounts payable	$ 5,871	$ 2,498
Accrued and other current liabilities	30,093	18,567
Deferred revenue - current portion	5,763	18,991
Total current liabilities	$ 41,727	$ 40,056
Deferred revenue - net of current portion	8,558	—
Other long term liabilities	1,874	2,478
Total long term liabilities	10,432	2,478
Total liabilities	$ 52,159	$ 42,534
Stockholders' equity		
Common stock, par value of $0.0001 per share; 300,000,000 shares authorized, 55,120,002 and 54,032,826 shares issued and outstanding as of September 30, 2025 and December 31, 2024, respectively	5	5
Additional paid-in capital	747,435	719,997
Accumulated other comprehensive income (loss)	446	(151)
Accumulated deficit	(439,781)	(490,830)
Total stockholders' equity	$ 308,105	$ 229,021
Total liabilities and stockholders' equity	$ 360,264	$ 271,555

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

	Three Months Ended September 30,		Nine Months Ended September 30,	
	2025	2024	2025	2024
Revenue	$ 10,632	$ 4,894	$ 183,018	$ 13,941
Operating expenses:				

Research and development	37,696	22,205	96,227	65,710
Sales, general and administrative	16,027	12,692	45,942	35,950
Total operating expenses	53,723	34,897	142,169	101,660
Income (loss) from operations	(43,091)	(30,003)	40,849	(87,719)
Other income (expense):				
Interest income (expense), net	3,463	3,545	10,141	9,668
Other income (expense), net	3	28	59	(448)
Total other income (expense)	3,466	3,573	10,200	9,220
Income (loss) before income taxes	$ (39,625)	$ (26,430)	$ 51,049	$ (78,499)
Provision for income taxes	(1,278)	—	—	—
Net income (loss)	$ (38,347)	$ (26,430)	$ 51,049	$ (78,499)
Net income (loss) per share:				
Basic	$ (0.65)	$ (0.47)	$ 0.88	$ (1.48)
Diluted	(0.65)	(0.47)	0.85	(1.48)
Weighted-average common shares outstanding:				
Basic	58,611,677	56,341,074	58,278,812	52,991,015
Diluted	58,611,677	56,341,074	60,397,681	52,991,015
Comprehensive income (loss):				
Net income (loss)	$ (38,347)	$ (26,430)	$ 51,049	$ (78,499)
Other comprehensive gain (loss):				
Unrealized gain on marketable securities	350	181	597	190
Total other comprehensive gain	$ 350	$ 181	$ 597	$ 190
Comprehensive income (loss)	$ (37,997)	$ (26,249)	$ 51,646	$ (78,309)

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