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

August 3, 2026

– Phase 3 EMPEROR study of zorevunersen in Dravet syndrome: Enrollment of 162 patients complete with data readout anticipated in Q3 2027 –

– Pre-NDA meeting with FDA scheduled for H2 2026; rolling U.S. NDA submission planned to initiate in Q1 2027 –

– Phase 1 OSPREY study of STK-002 in patients with ADOA: Dosing complete in sentinel cohort; dose escalation continuing and initial data anticipated in H1 2027 –

– $420M in cash, cash equivalents and marketable securities based on $354.3M as of June 30, 2026 and $65.7M from a subsequent ATM sale; expected to fund operations through to potential U.S. commercialization of zorevunersen in early 2028 –

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

BEDFORD, Mass.--(BUSINESS WIRE)--Aug. 3, 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 as a first-in-class potential disease-modifying treatment for Dravet syndrome. The Company today reported financial results for the second quarter ended June 30, 2026, and provided business updates including progress of the global Phase 3 EMPEROR study, preparation for a rolling New Drug Application (NDA) submission to the U.S. Food and Drug Administration (FDA) and the advancement of STK-002 as a potential treatment for Autosomal Dominant Optic Atrophy (ADOA).

Stoke will participate in a pre-NDA meeting with the FDA during the second half of 2026 as the Company prepares to initiate the planned rolling NDA submission for zorevunersen in the first quarter of 2027. With the June 2026 completion of enrollment of 162 patients into EMPEROR, a Phase 3 readout is anticipated in the third quarter of 2027. These data are expected to complete the rolling U.S. NDA submission in the second half of 2027. Beyond zorevunersen, dose escalation is underway in the Phase 1 OSPREY study of STK-002 in patients with ADOA, the most common inherited optic nerve disorder. Safety and efficacy data are anticipated in the first half of 2027.

"We have made significant progress across our business this year, including the rapid enrollment of 162 patients into the Phase 3 EMPEROR study and progression of these patients through key study milestones with the first patients now approaching the end of the 52-week treatment period," said Ian F. Smith, Chief Executive Officer and Director of Stoke Therapeutics. "With no treatment discontinuations to date in EMPEROR, and supportive four-year safety and efficacy data from the ongoing open-label extension studies, we have confidence in the potential of zorevunersen to change the course of Dravet syndrome. We look forward to our upcoming pre-NDA meeting with the FDA to align on the overall data package content and timing, as well as the statistical analysis plan, as we prepare to initiate our rolling U.S. NDA submission in the first quarter of 2027."

Mr. Smith continued, "Beyond Dravet syndrome, we are advancing our pipeline. Dose escalation continues in our Phase 1 study of STK-002 in patients with ADOA and initial data are anticipated in the first half of 2027 to guide future clinical development. We are also expanding our early research efforts to identify new haploinsufficient disease targets, and we recently strengthened our leadership team with the addition of Tom McCauley as Chief Scientific Officer to help guide this next phase of platform expansion and translational research. In parallel we continue to enhance our commercial capabilities, enabled by our strong financial position of approximately $420 million that will take us through to a potential U.S. launch of zorevunersen by early 2028 and support the Company's next phase of growth."

Program Highlights

Dravet syndrome (zorevunersen)

- **Pivotal Phase 3 EMPEROR study progress:**
  - **U.S., UK and Japan:** In June, Stoke announced completion of enrollment of 162 patients in the U.S., UK and Japan where sham control is administered via lumbar puncture (LP). This is the planned primary analysis population for the U.S. NDA. Patients are consistently progressing through the study. As of July 31, 2026:
    - Approximately 145 are through Week 8 and have received either two 70 mg loading doses of zorevunersen or sham.
    - Approximately 80 are through Week 24 and therefore have one dose of zorevunersen or sham remaining in the 52-week treatment period.
    - Approximately 60 are through Week 28, the time point at which the primary endpoint of change in major motor seizure frequency will be measured.
    - The first patients are expected to reach Week 52 in August 2026.
    - No patients have discontinued treatment in the study.

    - Data from this cohort are anticipated in the third quarter of 2027 and are expected to be the final clinical data required to complete the planned rolling NDA submission to the FDA. Stoke expects a pre-NDA meeting with the FDA in the second half of 2026 and to initiate the rolling NDA process in the first quarter of 2027, with completion expected in the second half of 2027.
    - **Europe (Germany, France, Spain and Italy):** An additional cohort of approximately 30 patients is currently being enrolled in Europe, where the sham control is administered via a needle prick (NP). Screening for this cohort has now closed, and the last patient is expected to be enrolled in August 2026.
    - **China:** Site activation and screening are also underway in China, where zorevunersen was recently granted Breakthrough Therapy Designation. Enrollment is anticipated to complete in the second half of 2026. Data from the additional patients in Europe and China are not planned for inclusion in the U.S. NDA submission to the FDA.
- **Continuing awareness of Dravet syndrome and appreciation for zorevunersen with 5 years of clinical data:**
    - The Company will continue its educational efforts at major neurology and epilepsy congresses during the second half of 2026. Analyses of data from the ongoing OLEs, including effects on seizure severity, improvements in seizure freedom and quality of life, will support efforts to increase clinician understanding of zorevunersen while the Phase 3 EMPEROR study advances toward completion and the Company prepares for a potential U.S. approval and launch. The Company will also continue discussions with the FDA to update the Agency on its long-term data for zorevunersen.

Pipeline beyond zorevunersen

- The Phase 1 OSPREY study of STK-002 for the treatment of ADOA is continuing through dose escalation cohorts. All eight planned clinical trial sites are now active in the UK, Germany, Denmark, Italy and Austria, and dosing of the first cohort of patients (n=3) is complete with no serious or severe safety events observed to date. Dosing of the second cohort is expected to begin in August 2026. Subject to ongoing safety assessments, dosing in the third and fourth dose cohorts are expected to follow with a readout of safety and efficacy results anticipated in the first half of 2027.
- Lead optimization is underway to identify a clinical candidate for the treatment of SYNGAP1-related disorders. SYNGAP1-related disorders are severe and rare neurodevelopmental diseases.
- Stoke is expanding its early research efforts with new targets in haploinsufficient diseases, primarily focused on central nervous system (CNS) diseases.
- Thomas McCauley, Ph.D., joined Stoke as Chief Scientific Officer in July to support this pipeline growth, leveraging the Company’s proprietary RNA medicines platform to advance its pipeline of potential treatments for severe genetic diseases.

Second quarter 2026 financial results

- The Company has $420.0 million in cash, cash equivalents and marketable securities based on $354.3 million as of June 30, 2026 and $65.7 million in net proceeds generated from an ATM sale to a single investor after June 30, 2026. These funds are expected to support operations through to potential U.S. commercialization of zorevunersen in early 2028.
- Revenue recognized for the three months ended June 30, 2026, was $9.3 million, a decrease from $13.8 million for the same period in 2025. Revenue is generated from satisfying contractual obligations of the collaboration and licensing agreements with Acadia and Biogen.
- Net loss for the three months ended June 30, 2026, was $61.6 million (including non-cash stock-based compensation expense of $10.8 million), or $0.93 per share, compared to a net loss of $23.5 million (including non-cash stock-based compensation expense of $7.6 million), or $0.40 per share, for the same period in 2025.
- Research and development expenses for the three months ended June 30, 2026, were $49.5 million, compared to $25.9 million for the same period in 2025. The increase was driven by increased activities and personnel expenses to support the advancement of zorevunersen.
- Sales, general and administrative expenses for the three months ended June 30, 2026, increased to $25.3 million from $15.3 million for the same period in 2025. The increase was driven by growth in personnel and launch readiness expenses.

Year-to-Date 2026 Financial Results

- Revenue recognized for the six months ended June 30, 2026, was $15.6 million, a decrease from $172.4 million for the same period in 2025. The decrease in revenue is primarily driven by the recognition of $150.8 million related to the Biogen IP license performance obligation during the six months ended June 30, 2025.
- Net loss for the six months ended June 30, 2026, was $111.6 million (including non-cash stock-based compensation expense of $19.6 million), or $1.73 per share, compared to a net income of $89.4 million (including non-cash stock-based compensation expense of $14.4 million), or $1.50 per diluted share, for the same period in 2025.
- Research and development expenses for the six months ended June 30, 2026, were $89.2 million, compared to $58.5 million for the same period in 2025. The increase was driven by increased activities and personnel expenses to support the advancement of zorevunersen.
- Sales, general and administrative expenses for the six months ended June 30, 2026, increased to $45.2 million from $29.9

million for the same period in 2025. The increase was driven by growth 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 Monday, August 3, 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, and China's Center for Drug Evaluation has granted zorevunersen Breakthrough Therapy Designation. Stoke has a strategic collaboration with Biogen (Nasdaq: BIIB) 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 completed enrollment of 162 patients in the United States, United Kingdom and Japan in June 2026, and a data readout is anticipated in the third quarter of 2027 to support the submission of a rolling New Drug Application (NDA) to the FDA. Approximately 30 additional patients are expected to enroll in Germany, Spain, France and Italy, with completion of enrollment anticipated in August 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 open-label period of the 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 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 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. Dosing of the first cohort of patients (n=3) is complete, and dosing of the second cohort is expected to begin in August 2026. Subject to ongoing safety assessments, dosing in the third and fourth cohorts are expected to follow with a readout of safety and efficacy results anticipated in the first half of 2027. Data from the OSPREY study will help to inform potential future development of STK-002.

All eight planned OSPREY clinical trial sites are now active in the UK, Germany, Denmark, Italy and Austria. 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/ or follow us on LinkedIn.

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 potential benefits, safety and efficacy of zorevunersen; 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 Company's ability to achieve an NDA submission or approval on the expedited timeframe disclosed or at all; 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 potential 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 Company's ability to protect its intellectual property; the Company's ability to fund development activities and achieve development goals into 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.
Condensed consolidated balance sheets
(in thousands, except share and per share amounts)

	June 30, 2026	December 31, 2025
Assets		
Current assets:		
Cash and cash equivalents	$ 110,004	$ 84,220
Marketable securities - current	182,814	200,450
Accounts receivable	7,179	5,936
Prepaid expenses	14,688	8,736
Interest receivable	1,540	1,969
Other current assets	6,313	4,389
Total current assets	$ 322,538	$ 305,700

Marketable securities - long-term	61,502	106,260
Restricted cash - long-term	3,227	227
Operating lease right-of-use assets	1,870	3,101
Property and equipment, net	4,829	3,146
Total assets	$ 393,966	$ 418,434
Liabilities and stockholders' equity		
Current liabilities:		
Accounts payable	$ 4,794	$ 4,939
Accrued and other current liabilities	28,613	41,035
Deferred revenue - current portion	8,523	11,901
Total current liabilities	$ 41,930	$ 57,875
Deferred revenue - net of current portion	5,652	6,961
Other long-term liabilities	837	1,141
Total long-term liabilities	6,489	8,102
Total liabilities	$ 48,419	$ 65,977
Stockholders' equity		
Common stock, par value of $0.0001 per share; 300,000,000 shares authorized, 62,402,595 and 58,921,999 shares issued and outstanding as of June 30, 2026 and December 31, 2025, respectively	6	5
Additional paid-in capital	955,419	849,624
Accumulated other comprehensive (loss) income	(542)	543
Accumulated deficit	(609,336)	(497,715)
Total stockholders' equity	$ 345,547	$ 352,457
Total liabilities and stockholders' equity	$ 393,966	$ 418,434

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

	Three Months Ended June 30,		Six Months Ended June 30,	
	2026	2025	2026	2025
Revenue	$ 9,325	$ 13,817	$ 15,554	$ 172,386
Operating expenses:				
Research and development	49,501	25,855	89,174	58,531
Sales, general and administrative	25,253	15,262	45,227	29,915
Total operating expenses	74,754	41,117	134,401	88,446
(Loss) income from operations	(65,429)	(27,300)	(118,847)	83,940
Other income (expense):				
Interest income (expense), net	3,503	3,789	6,899	6,678
Other income	308	28	327	57
Total other income (expense)	3,811	3,817	7,226	6,735
(Loss) income before income taxes	$ (61,618)	$ (23,483)	$ (111,621)	$ 90,675
Provision for income taxes	—	—	—	1,278
Net (loss) income	$ (61,618)	$ (23,483)	$ (111,621)	$ 89,397
Net (loss) income per share:				
Basic	$ (0.93)	$ (0.40)	$ (1.73)	$ 1.54
Diluted	(0.93)	(0.40)	(1.73)	1.50
Weighted-average common shares outstanding:				
Basic	66,017,895	58,353,855	64,548,862	58,109,622
Diluted	66,017,895	58,353,855	64,548,862	59,681,472
Comprehensive (loss) income:				
Net (loss) income	$ (61,618)	$ (23,483)	$ (111,621)	$ 89,397
Other comprehensive (loss) gain:				
Unrealized (loss) gain on marketable securities	(427)	200	(1,085)	247
Total other comprehensive (loss) gain	$ (427)	$ 200	$ (1,085)	$ 247
Comprehensive (loss) income	$ (62,045)	$ (23,283)	$ (112,706)	$ 89,644

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/20260803082079/en/

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