



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

October 29, 2026

–Phase 3 EMPEROR study of zorevunersen, an investigational medicine for Dravet syndrome: ~110 patients through Week 28 primary endpoint, ~20 patients through Week 52 and all continuing into open-label treatment period; data readout anticipated in Q3 2027 followed by the planned U.S. NDA submission in H2 2027–

–Successful FDA meeting to align on U.S. NDA submission: Changes in cognition and behavior, a key secondary endpoint in EMPEROR, will be analyzed using a multicomponent assessment of four Vineland-3 subdomains–

–STK-002, an investigational medicine for ADOA: Dose escalation in the Phase 1 OSPREY study continues with dosing of third cohort underway; initial safety and efficacy data anticipated in H1 2027–

–\$358.8M in cash, cash equivalents and marketable securities as of September 30, 2026 expected to fund operations into early 2028–

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

BEDFORD, Mass.--(BUSINESS WIRE)--Oct. 29, 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 third quarter ended September 30, 2026, and provided business updates including progress of the global Phase 3 EMPEROR study, ongoing preparations to support regulatory submissions for zorevunersen in the U.S., and the advancement of STK-002 as a potential treatment for people with Autosomal Dominant Optic Atrophy (ADOA).

"The Phase 3 EMPEROR study of zorevunersen continues to progress well. Approximately 110 of 162 enrolled patients have completed their Week 28 visit when the primary endpoint of change in major motor seizure frequency is measured and approximately 20 are through Week 52, the end of the treatment period. All 20 of these patients are continuing into the open-label extension period of the study and to date, no patients have discontinued treatment," said Ian F. Smith, Chief Executive Officer and Director of Stoke Therapeutics. "Our NDA planning is on track following our recent discussion with the FDA. Importantly, the key secondary endpoint of changes in patients' cognition and behavior will be measured using a multicomponent assessment of four Vineland-3 subdomains. This multicomponent measure reflects the diversity of effects that people with Dravet syndrome experience and how to best evaluate them in a clinical trial. We look forward to the Phase 3 data readout in the third quarter of 2027 and completing our NDA submission in the second half of 2027."

Mr. Smith continued, "Our second clinical program, STK-002, targets the protein haploinsufficiency that causes ADOA and therefore may have the potential to improve vision in people living with this severe genetic disease that often leads to blindness. Dosing in the first two cohorts is complete and dosing in the third cohort is underway with the fourth cohort expected to follow in early 2027. Safety and efficacy data are anticipated in the first half of 2027. With this progress across the business, we are building Stoke for the future with the team and capabilities to realize the full potential of zorevunersen, our pipeline and our unique scientific approach."

Program Highlights

Dravet syndrome (zorevunersen)

- **Pivotal Phase 3 EMPEROR Study Progress:** Global enrollment is now complete. Enrollment of 162 patients in the primary analysis population (the United States, United Kingdom and Japan) was completed in June 2026, and a data readout from these patients is anticipated in the third quarter of 2027. Patients are progressing through the study and as of October 29, 2026:
 - **All 162 patients** are through Week 8 and have received either two 70 mg loading doses of zorevunersen or sham.
 - **Approximately 130** are through Week 24 and therefore have completed three of the four doses of zorevunersen or sham in the 52-week treatment period.
 - **Approximately 110** are through Week 28, the time point at which the primary endpoint of change from baseline in log-transformed major motor seizure frequency is measured.
 - **Approximately 65** are through Week 40 and have therefore completed all doses of zorevunersen or sham in the 52-week treatment period.
 - **Approximately 20** are through Week 52, the end of the treatment period. All of these patients are continuing into the open-label treatment period.
 - **No patients** have discontinued treatment in the study.

- **Successful Meeting with the FDA:** Stoke recently met with the U.S. Food and Drug Administration (FDA) to align on key aspects of the planned U.S. New Drug Application (NDA) for zorevunersen. Stoke plans to continue actively engaging with the FDA and to submit data throughout the first half of 2027 to support rapid completion of the NDA submission in the second half of 2027. An important outcome from the discussion included:
 - **New Multicomponent Assessment:** For the NDA, Stoke plans to use a multicomponent assessment of four prespecified Vineland-3 subdomains to evaluate changes in cognition and behavior in the Phase 3 EMPEROR study. These four subdomains, expressive communication, receptive communication, interpersonal relationships and personal skills, were selected based on feedback from clinicians and caregivers and their potential to demonstrate meaningful effects for patients within the 52-week treatment period where patients receive zorevunersen or sham. The treatment effect for each subdomain will be calculated using a multivariate mixed model for repeated measures, or MMRM. These scores will then be averaged to produce a single score that reflects the overall treatment effect on cognition and behavior.
- **Additional EMPEROR Study Patients:** Enrollment of 34 patients in Europe (Germany, France, Spain and Italy) was completed in August 2026. Enrollment of 5 patients in China was completed in October 2026.
- **Continuing awareness of Dravet syndrome and appreciation for 5 years of clinical data for zorevunersen:**
 - Stoke and Biogen recently presented [long-term clinical data](#) that supported the disease-modifying potential of zorevunersen at the 16th European Epilepsy Congress (EEC) and the 55th Child Neurology Society (CNS) Annual Meeting. These data included a new exploratory sub-analysis that showed substantial reductions in the most severe seizure types, which are the leading risk factor for sudden unexpected death in epilepsy (SUDEP).
 - The Company is continuing its educational efforts at major neurology and epilepsy congresses through the remainder of 2026 and into 2027. Analyses of data 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 in early 2028.

Pipeline beyond zorevunersen

- The Phase 1 OSPREY study of STK-002 in people with ADOA is continuing through dose escalation. Dosing is complete in the first two cohorts, with dosing of the third cohort recently initiated and expected to complete by the end of 2026. Cohorts three and four have the potential to reach therapeutic levels of dosing, with initial safety and efficacy data from these latter cohorts anticipated in the first half of 2027.
 - Preclinical data and progress of the Phase 1 OSPREY study were [recently presented](#) at the American Academy of Ophthalmology (AAO) 2026 Annual Meeting. Presentations connected insights from ADOA disease biology and natural history findings with preclinical proof-of-concept data, supporting the disease-modifying potential of STK-002 in ADOA.
- Lead optimization is underway to identify a development candidate for the treatment of SYNGAP1-related disorders. SYNGAP1-related disorders are rare and often severe neurodevelopmental diseases.

Third Quarter 2026 Financial Results

- The Company has \$358.8 million in cash, cash equivalents and marketable securities as of September 30, 2026. This cash position is expected to fund operations into early 2028.
- Revenue recognized for the three months ended September 30, 2026, was \$11.3 million, an increase from \$10.6 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 September 30, 2026, was \$73.6 million (including non-cash stock-based compensation expense of \$11.0 million), or \$1.08 per share, compared to a net loss of \$38.3 million (including non-cash stock-based compensation expense of \$8.0 million), or \$0.65 per share, for the same period in 2025.
- Research and development expenses for the three months ended September 30, 2026, were \$59.8 million, compared to \$37.7 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 September 30, 2026, increased to \$28.7 million from \$16.0 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 nine months ended September 30, 2026, was \$26.8 million, a decrease from \$183.0 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 nine months ended September 30, 2025.
- Net loss for the nine months ended September 30, 2026, was \$185.3 million (including non-cash stock-based compensation expense of \$30.6 million), or \$2.81 per share, compared to a net income of \$51.0 million (including non-cash stock-based compensation expense of \$22.4 million), or \$0.85 per diluted share, for the same period in 2025.

- Research and development expenses for the nine months ended September 30, 2026, were \$149.0 million, compared to \$96.2 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 nine months ended September 30, 2026, increased to \$74.0 million from \$45.9 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 Thursday, October 29, 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 genetic 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% 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 infections¹. 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 Japan². There are no approved disease-modifying therapies for people living with Dravet syndrome.

About Zorevunersen

Zorevunersen is an investigational antisense oligonucleotide (ASO) 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 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. Enrollment in the United States, United Kingdom and Japan was completed in June 2026, and a data readout is anticipated in the third quarter of 2027. Stoke plans to submit an NDA to the FDA in the second half of 2027. Additional cohorts in Europe (Germany, France, Spain and Italy; 34 patients) and China (5 patients) completed enrollment in August and October 2026, respectively.

Participants are randomized 1:1 to receive either zorevunersen via intrathecal administration or a sham comparator for a 52-week treatment period following a baseline period. The primary endpoint in the U.S. is change from baseline in log-transformed major motor seizure frequency at Week 28, although all study data will be analyzed at Week 52. The first key secondary endpoint is the durability of effect on major motor seizure frequency at Week 52. The second key secondary endpoint is change from baseline in adaptive functioning (cognition and behavior) at Week 52. In the U.S., an analysis will use a multicomponent assessment that simultaneously evaluates four prespecified Vineland-3 subdomains: expressive communication, receptive communication, interpersonal relationships and personal skills. Additional endpoints include safety, Clinician Global Impression of Change (CGI-C), Caregiver Global Impression of Change (CaGI-C), Bayley Scales of Infant Development (BSID-IV) and EuroQol Visual Analog Scale (EQ-VAS). 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.

About Autosomal Dominant Optic Atrophy (ADOA)

ADOA is the most common inherited optic nerve disorder, affecting approximately one in 25,000 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. 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, of which more than 600 have been reported in people diagnosed with ADOA. Most of these variants in the *OPA1* gene 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 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 ongoing.

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 two cohorts of patients is complete, with dosing of the third cohort expected to complete by the end of 2026. Subject to ongoing safety assessments, dosing of the fourth cohort is expected to follow in early 2027. Initial safety and efficacy results are 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 Company's ability to achieve an NDA submission or approval on the expedited timeframe disclosed or at all, and the associated timing, if any, of a potential U.S. commercial launch; the ability of STK-002 to treat the underlying causes of Autosomal Dominant Optic Atrophy (ADOA) and 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)

	September 30, 2026	December 31, 2025
Assets		
Current assets:		
Cash and cash equivalents	\$ 107,271	\$ 84,220
Marketable securities - current	211,916	200,450
Accounts receivable	8,543	5,936
Prepaid expenses	17,596	8,736
Interest receivable	1,533	1,969
Other current assets	7,558	4,389

Total current assets	\$	354,417	\$	305,700
Marketable securities - long-term		39,602		106,260
Restricted cash - long-term		3,227		227
Operating lease right-of-use assets		1,233		3,101
Property and equipment, net		8,213		3,146
Total assets	\$	406,692	\$	418,434
Liabilities and stockholders' equity				
Current liabilities:				
Accounts payable	\$	4,029	\$	4,939
Accrued and other current liabilities		38,584		41,035
Deferred revenue - current portion		6,364		11,901
Total current liabilities	\$	48,977	\$	57,875
Deferred revenue - net of current portion		4,446		6,961
Other long-term liabilities		681		1,141
Total long-term liabilities		5,127		8,102
Total liabilities	\$	54,104	\$	65,977
Stockholders' equity				
Common stock, par value of \$0.0001 per share; 300,000,000 shares authorized, 64,813,537 and 58,921,999 shares issued and outstanding as of September 30, 2026 and December 31, 2025, respectively		6		5
Additional paid-in capital		1,036,612		849,624
Accumulated other comprehensive (loss) income		(1,050)		543
Accumulated deficit		(682,980)		(497,715)
Total stockholders' equity	\$	352,588	\$	352,457
Total liabilities and stockholders' equity	\$	406,692	\$	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 September 30,		Nine Months Ended September 30,	
	2026	2025	2026	2025
Revenue	\$ 11,255	\$ 10,632	\$ 26,808	\$ 183,018
Operating expenses:				
Research and development	59,831	37,696	149,004	96,227
Sales, general and administrative	28,739	16,027	73,966	45,942
Total operating expenses	88,570	53,723	222,970	142,169
(Loss) income from operations	(77,315)	(43,091)	(196,162)	40,849
Other income (expense):				
Interest income (expense), net	3,665	3,463	10,564	10,141
Other income	6	3	333	59
Total other income (expense)	3,671	3,466	10,897	10,200
(Loss) income before income taxes	\$ (73,644)	\$ (39,625)	\$ (185,265)	\$ 51,049
Provision for income taxes	—	(1,278)	—	—
Net (loss) income	\$ (73,644)	\$ (38,347)	\$ (185,265)	\$ 51,049
Net (loss) income per share:				
Basic	\$ (1.08)	\$ (0.65)	\$ (2.81)	\$ 0.88
Diluted	(1.08)	(0.65)	(2.81)	0.85
Weighted-average common shares outstanding:				
Basic	68,362,880	58,611,677	65,834,172	58,278,812
Diluted	68,362,880	58,611,677	65,834,172	60,397,681
Comprehensive (loss) income:				
Net (loss) income	\$ (73,644)	\$ (38,347)	\$ (185,265)	\$ 51,049
Other comprehensive (loss) gain:				
Unrealized (loss) gain on marketable securities	(508)	350	(1,593)	597

Total other comprehensive (loss) gain	\$	(508)	\$	350	\$	(1,593)	\$	597
Comprehensive (loss) income	\$	(74,152)	\$	(37,997)	\$	(186,858)	\$	51,646

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](#).

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