Stoke Therapeutics Announces Updates to Timelines for the Completion of Enrollment and a Phase 3 Data Readout from the EMPEROR Study of Zorevunersen for the Treatment of Dravet Syndrome

January 11, 2026

— Company now expects to complete enrollment of 150 patients in Q2 2026, with a Phase 3 data readout in mid-2027; Rolling NDA submission planned to initiate in first half 2027—

— Discussions with FDA ongoing following recent multidisciplinary meeting; Company continues to explore potential for expedited development, registration and delivery of zorevunersen to patients —

—Company to present at 44th Annual J.P. Morgan Healthcare Conference on Tuesday, January 13, 2026 —

BEDFORD, Mass.--(BUSINESS WIRE)--Jan. 11, 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. Today, the Company announced accelerated timelines for the completion of enrollment and a Phase 3 data readout from the EMPEROR study. Completion of enrollment of 150 patients is now expected in the second quarter of 2026. This enrollment progress puts the Phase 3 EMPEROR study on track for a data readout in mid-2027 that is anticipated to support the submission of a New Drug Application (NDA) to the U.S. Food and Drug Administration (FDA). The Company plans to initiate a rolling NDA submission in the first half of 2027.

In addition, as part of zorevunersen's Breakthrough Therapy Designation, a multidisciplinary meeting was held with the FDA to discuss the ongoing clinical development of zorevunersen, including the exploration of potential expedited regulatory pathways. No immediate changes to the zorevunersen development program were agreed to at the meeting. The FDA has requested additional information, and discussions between the Company and the Agency are ongoing regarding potential opportunities to expedite the development, registration and delivery of zorevunersen to patients.

"The rate of enrollment in the Phase 3 EMPEROR study is highly encouraging and supports the significant need for a disease-modifying treatment for Dravet syndrome. The accelerated timing for completion of enrollment of 150 patients, in addition to Breakthrough Therapy Designation, positions us to initiate a rolling NDA submission in the first half of 2027 resulting in the potential to deliver zorevunersen to patients sooner than originally expected," said Ian F. Smith, Chief Executive Officer and Director of Stoke Therapeutics. "Our recent multidisciplinary meeting was productive and we appreciate the FDA's interest in deepening their understanding of Dravet syndrome and its impacts on patients and their families while also taking time to review and discuss our four years of clinical data. Subsequently, we have responded to the Agency's request for additional information and we look forward to continuing to engage with them as part of our commitment to explore every opportunity to deliver zorevunersen to patients as quickly as possible."

As of January 9, 2026, nearly 330 patients globally had been identified by investigators as potential study candidates. Of these, approximately 60 patients are currently in the formal 8-week screening period that immediately precedes randomization and an additional approximately 60 patients have advanced to randomization and dosing.

Financial Guidance

The Company has approximately $391.7 million in cash, cash equivalents and marketable securities as of December 31, 2025. These funds, combined with eligible proceeds from the Biogen collaboration, are anticipated to fund operations into 2028.

J.P. Morgan Healthcare Conference Webcast Information

Chief Executive Officer Ian F. Smith will present at the 44th Annual J.P. Morgan Healthcare Conference on Tuesday, January 13, 2026, at 7:30 p.m. Eastern Time (4:30 p.m. Pacific Time) in San Francisco, CA. A live audio webcast of the presentation will be available on the Investors & News section of Stoke's website at https://investor.stoketherapeutics.com/ and can be accessed by following this Link. A replay of the webcast will be available for 30 days following the presentation.

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 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. The trial is currently enrolling approximately 150 patients in the United States, United Kingdom and Japan which are anticipated to support an NDA. At least 20 additional patients are expected to enroll in Germany, Spain, France and Italy starting in the second quarter of 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://www.emperorstudy.com/.

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/.

Stoke Therapeutics 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 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 timing and expected progress of clinical trials, data readouts, regulatory meetings, regulatory decisions and other presentations; the characterization of the Company's meeting and discussions with the FDA; and the ability of the Company to achieve an NDA submission or approval on the expedited timeframe disclosed or at all. Statements including words such as "plan," "potential," "will," "continue," "expect," or similar words 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 Stoke'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 and ultimately commercialize its product candidates; that if collaborators were to breach or terminate their agreements, 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; 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 its 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.

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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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

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