



Stoke Therapeutics Announces Completion of Successful Meeting with the FDA to Align on Planned U.S. NDA for Zorevunersen, an Investigational Medicine for the Treatment of Dravet Syndrome

October 5, 2026

– Analysis of the Phase 3 EMPEROR key secondary endpoint of changes in cognition and behavior will be performed using a multicomponent assessment of four Vineland-3 subdomains –

– Phase 3 readout anticipated in the third quarter of 2027 to complete the planned U.S. NDA submission in the second half of 2027 –

BEDFORD, Mass.--(BUSINESS WIRE)--Oct. 5, 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 announced the completion of a successful meeting with the U.S. Food and Drug Administration (FDA) intended to align on key aspects of the Company's planned submission of a New Drug Application (NDA) for zorevunersen. The meeting provided an opportunity to discuss the 5 years of clinical safety and efficacy data from the Phase 1/2a and ongoing open-label extension (OLE) studies of zorevunersen and to obtain feedback on the planned NDA submission, including timing, data to be included and the analysis of Vineland-3 data, a key secondary endpoint in the Phase 3 EMPEROR study.

Following the discussion with the FDA, Stoke now 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 one-year EMPEROR treatment period. For purposes of the U.S. NDA submission, Vineland-3 subdomain data will be analyzed using a multivariate mixed model for repeated measures (MMRM).

The Company 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 following the Phase 3 readout anticipated in the third quarter of 2027. With these data, the Company plans to complete the U.S. NDA submission in the second half of 2027 to support a potential U.S. approval and launch of zorevunersen in early 2028.

"We are encouraged by our productive dialogue with the FDA and the Agency's deepening understanding of the severity of Dravet syndrome and need for better treatments, and 5 years of safety and efficacy data for zorevunersen," said Ian F. Smith, Chief Executive Officer and Director of Stoke Therapeutics. "We are pleased with the Agency's willingness to accept a multicomponent assessment of Vineland-3 data measuring changes in cognition and behavior from patients treated in the Phase 3 EMPEROR study. We believe this is the most appropriate way to capture the effects of zorevunersen on people with Dravet syndrome who experience a wide range of debilitating neurodevelopmental impacts. We look forward to continuing our interactions with the FDA as we advance the EMPEROR study and prepare for the readout of data in the third quarter of 2027, followed quickly by the completion of our NDA submission in the second half of 2027."

Phase 3 EMPEROR Study Design and Evaluation of Endpoints

Participants in EMPEROR 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 is change from baseline in log-transformed major motor seizure frequency at Week 28, although all study data will be analyzed at Week 52.

In the U.S., a MMRM analysis will be used to estimate the treatment effect of zorevunersen compared to sham using data collected from baseline through Week 52. The primary treatment effect is the estimated change in seizure frequency from baseline at Week 28, for the 4-week interval immediately preceding Week 28 (Week 24 to Week 28).

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 (MMRM) that simultaneously evaluates four prespecified Vineland-3 subdomains: expressive communication, receptive communication, interpersonal relationships and personal skills. All data will be analyzed at Week 52.

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).

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 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. Stoke completed enrollment in the United States, United Kingdom and Japan in June 2026, and a data readout is anticipated in the third quarter of 2027 to complete the planned submission of a New Drug Application (NDA) to the FDA. An additional cohort of 34 patients in Europe (Germany, France, Spain and Italy) completed enrollment in August 2026. Enrollment is currently underway in China and is anticipated to complete in the second half of 2026. 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 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](#).

Stoke 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 design, timing and expected progress of clinical trials, data readouts, regulatory meetings, regulatory decisions and other presentations; and the potential timing for the submission of data to the FDA, the completion of the U.S. NDA, and the potential U.S. approval and launch of zorevunersen. 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 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 and ultimately commercialize its product candidates; that if the Company's 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 its collaborator 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; Stoke's ability to protect its intellectual property; Stoke'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, 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 Stoke 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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