



Stoke Therapeutics and Biogen Present Long-Term Clinical Data that Support the Disease-Modifying Potential of Zorevunersen, an Investigational Medicine for the Treatment of Dravet Syndrome, at the 16th European Epilepsy Congress (EEC)

September 3, 2026

–4-year data from the Phase 1/2a open-label extension (OLE) studies showed substantial and durable reductions in seizures and continuing improvements in cognition and behavior in patients treated with zorevunersen on top of standard of care anti-seizure medicines–

–New data showed substantial reductions in the most severe seizure types, the leading risk factor for sudden unexpected death in epilepsy (SUDEP)–

–Improvements in quality of life were demonstrated through 28 months of treatment–

–Zorevunersen generally well tolerated, with some patients treated for more than 5 years–

–Data from the global, pivotal Phase 3 EMPEROR study anticipated in Q3 2027 to complete the planned rolling U.S. NDA submission to the FDA–

BEDFORD, Mass. and CAMBRIDGE, Mass., Sept. 03, 2026 (BUSINESS WIRE) -- [Stoke Therapeutics, Inc.](#) (Nasdaq: STOK), a biotechnology company dedicated to restoring protein expression by harnessing the body's potential with RNA medicine, and [Biogen Inc.](#) (Nasdaq: BIIB) today announced presentations of data at the 16th European Epilepsy Congress (EEC), taking place September 5-9 in Athens, Greece. These data support the potential of zorevunersen as a first-in-class disease-modifying treatment for Dravet syndrome. Dravet syndrome is a severe developmental and epileptic encephalopathy (DEE) characterized by recurrent seizures as well as significant cognitive and behavioral impairments.

Data presented at EEC represent more than 5 years of clinical experience with zorevunersen in patients with Dravet syndrome, including two Phase 1/2a and ongoing open-label extension studies (OLEs). Four-year OLE results showed substantial and durable reductions in seizures and continuing improvements in cognition and behavior. A new exploratory sub-analysis also showed substantial reductions in the most severe seizure types, which are the leading risk factor for sudden unexpected death in epilepsy (SUDEP)¹. SUDEP is the primary cause of premature death in Dravet syndrome², and up to 20% of children and adolescents with the disease die before reaching adulthood³. An additional sub-analysis presented at EEC demonstrated substantial improvements in quality of life through 28 months in the OLEs. Zorevunersen continues to be generally well tolerated in the OLEs.

"Seizures are the most acute symptom of Dravet syndrome but the disease affects nearly every aspect of a child's development, from their ability to communicate with loved ones to skills like dressing and feeding themselves," said Helen Cross, MB ChB, Ph.D., Professor, The Prince of Wales's Chair of Childhood Epilepsy and Director of University College London Great Ormond Street Institute of Child Health, Honorary Consultant in Paediatric Neurology at Great Ormond Street Hospital. "The continuing improvements in cognition and behavior shown in these studies suggest zorevunersen has the potential to narrow the developmental gap between these children and their neurotypical peers, helping them gain more independence and participate in experiences that many thought might never be possible. Taken together, the data from studies of zorevunersen offer hope for a very different future for people living with Dravet syndrome and their families."

The global, pivotal Phase 3 EMPEROR study is underway to evaluate the efficacy and safety of zorevunersen. Enrollment has completed in the planned primary analysis population, which will evaluate zorevunersen compared to sham administered via lumbar puncture (LP) in 162 patients enrolled in the U.S., U.K. and Japan. A Phase 3 data readout is anticipated in the third quarter of 2027 to complete the planned rolling New Drug Application (NDA) to the U.S. Food and Drug Administration (FDA) in the second half of 2027. Enrollment in Europe has also completed with 34 participants enrolled.

Summary of Data from the Phase 1/2a and OLE Studies Presented at EEC

- **4-year OLE data:** Following treatment in the Phase 1/2a studies, 93% (75/81) of eligible patients continued treatment in the OLEs. As of the 4-year data cutoff, 77% (58/75) of patients remained in these studies. Patients receiving zorevunersen on top of standard anti-seizure medicines (ASMs) continued to experience durable reductions in seizures and ongoing improvements in cognition and behavior. Statistically significant improvements in cognition and behavior were demonstrated at 1, 2, 3 and 4 years of treatment compared to OLE baseline.
- **Severe seizure analysis:** Patients with Dravet syndrome experience frequent, prolonged and refractory seizures. Compared with the general epilepsy population, patients with Dravet syndrome have a significantly increased risk of SUDEP². Seizures are classified based on severity, with generalized tonic-clonic (GTC), focal-to-bilateral tonic-clonic (focal-to-BTC) and tonic-clonic seizures of unknown origin considered the most severe and correlated with the highest morbidity and mortality in people with epilepsy¹. Substantial reductions in GTC and focal-to-BTC seizures were demonstrated

through 3 years of the OLEs, compared to Phase 1/2a baseline, in patients treated with zorevunersen on top of standard of care ASMs.

- **Quality of life analysis:** Patients experienced substantial improvements in quality of life through 28 months in the OLEs, compared to Phase 1/2a baseline, as measured by EuroQol Visual Analog Scale (EQ-VAS, a component of the Euro-Qol-5D Youth). EQ-VAS is a validated measure of overall health status ranging from 0 to 100 (worst to best imaginable health) and provides insight into the real-world impact of zorevunersen on patients with Dravet syndrome and their families.

“Up to 20% of children and adolescents with Dravet syndrome die before reaching adulthood, and SUDEP is the primary cause,” said Barry Ticho, M.D., Ph.D., Chief Medical Officer of Stoke Therapeutics. “These data are especially meaningful because they show substantial reductions in the severe seizures most strongly correlated with SUDEP and demonstrate continuing improvements in the debilitating neurodevelopmental aspects of the disease. Together with ongoing improvements in quality of life, these data increase our confidence in what zorevunersen may one day deliver for the Dravet community.”

“For patients with a chronic disease like Dravet syndrome, safety and tolerability are critically important,” said Stephanie Fradette, Pharm.D., Head of the Rare Neurology Development Unit at Biogen. “The ongoing open-label extension studies will continue to grow the body of evidence shaping our understanding of zorevunersen’s long-term safety as well as its potential to address the underlying genetic cause of Dravet syndrome and improve outcomes for patients. We look forward to results from the Phase 3 EMPEROR study next year.”

Summary of Zorevunersen Safety Data

- Zorevunersen continues to be generally well tolerated, with some patients treated for more than 5 years in the Phase 1/2a and ongoing OLE studies. As of July 31, 2026, more than 930 doses have been administered.
- Elevated CSF protein lab values occurred in approximately 94% of patients, of which 59% have been classified as a treatment-emergent adverse event. Importantly, no serious or severe clinical manifestations have been associated with CSF protein elevations. There have been no reports of hydrocephalus.

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 infections³. 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 Japan⁴. 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. Results from the Phase 1/2a and OLE studies were published in [The New England Journal of Medicine \(NEJM\)](#) in March 2026. 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 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. Enrollment in Europe completed in August 2026. Enrollment is currently underway in China and is anticipated to complete in the second half of 2026. 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 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 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](#).

About Biogen

Founded in 1978, Biogen is a leading biotechnology company that pioneers innovative science to deliver new medicines to transform patients' lives and to create value for shareholders and our communities. We apply deep understanding of human biology and leverage different modalities to advance first-in-class treatments or therapies that deliver superior outcomes. Our approach is to take bold risks, balanced with return on investment to deliver long-term growth. We routinely post information that may be important to investors on our website at www.biogen.com. Follow us on social media - [Facebook](#), [Instagram](#), [LinkedIn](#), [X](#), [YouTube](#).

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 initiation and completion of the U.S. NDA submission to the FDA. 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: Stoke's ability to advance, obtain regulatory approval and ultimately commercialize its product candidates; that if Biogen were to breach or terminate the collaboration, Stoke would not obtain the anticipated financial or other benefits; the possibility that Stoke 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; 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.

Biogen Safe Harbor

This news release contains forward-looking statements, including, among others, relating to: the potential clinical effects of zorevunersen; the potential for zorevunersen to improve outcomes for patients with Dravet syndrome; the expected timing of Phase 3 study results; the potential benefits, safety and efficacy of zorevunersen; potential regulatory discussions, applications, submissions and approvals and the timing thereof; the potential treatment of the underlying genetic cause of Dravet syndrome; the anticipated benefits, risks and potential of Biogen's collaboration arrangements with Stoke Therapeutics; the potential of Biogen's commercial business and pipeline programs, including zorevunersen; and risks and uncertainties associated with drug development and commercialization. These forward-looking statements may be accompanied by such words as "aim," "anticipate," "assume," "believe," "contemplate," "continue," "could," "estimate," "expect," "forecast," "goal," "guidance," "hope," "intend," "may," "objective," "outlook," "plan," "possible," "potential," "predict," "project," "prospect," "should," "target," "will," "would" or the negative of these words or other words and terms of similar meaning. Drug development and commercialization involve a high degree of risk, and only a small number of research and development programs result in commercialization of a product. Results in early-stage clinical trials may not be indicative of full results or results from later stage or larger scale clinical trials and do not ensure regulatory approval. You should not place undue reliance on these statements. Given their forward-looking nature, these statements involve substantial risks and uncertainties that may be based on inaccurate assumptions and could cause actual results to differ materially from those reflected in such statements.

These forward-looking statements are based on management's current beliefs and assumptions and on information currently available to management. Given their nature, we cannot assure that any outcome expressed in these forward-looking statements will be realized in whole or in part. We caution that these statements are subject to risks and uncertainties, many of which are outside of our control and could cause future events or results to differ materially from those stated or implied in this document, including, among others, uncertainty of our long-term success in developing, licensing, or acquiring other product candidates or additional indications for existing products; expectations, plans, prospects and timing of actions relating to product approvals, approvals of additional indications for our existing products, sales, pricing, growth, reimbursement and launch of our marketed and pipeline products; the potential impact of increased product competition in the biopharmaceutical and healthcare industry, as well as any other markets in which we compete, including increased competition from new originator therapies, generics, prodrugs and biosimilars of existing

products and products approved under abbreviated regulatory pathways; our ability to effectively implement our corporate strategy; difficulties in obtaining and maintaining adequate coverage, pricing, and reimbursement for our products; the drivers for growing our business, including our dependence on collaborators and other third parties for the development, regulatory approval, and commercialization of products and other aspects of our business, which are outside of our full control; risks related to commercialization of biosimilars, which is subject to such risks related to our reliance on third-parties, intellectual property, competitive and market challenges and regulatory compliance; the risk that positive results in a clinical trial may not be replicated in subsequent or confirmatory trials or success in early stage clinical trials may not be predictive of results in later stage or large scale clinical trials or trials in other potential indications; risks associated with clinical trials, including our ability to adequately manage clinical activities, unexpected concerns that may arise from additional data or analysis obtained during clinical trials, regulatory authorities may require additional information or further studies, or may fail to approve or may delay approval of our drug candidates; and the occurrence of adverse safety events, restrictions on use with our products, or product liability claims; and any other risks and uncertainties that are described in other reports we have filed with the U.S. Securities and Exchange Commission, which are available on the SEC's website at www.sec.gov.

These statements speak only as of the date of this press release and are based on information and estimates available to us at this time. Should known or unknown risks or uncertainties materialize or should underlying assumptions prove inaccurate, actual results could vary materially from past results and those anticipated, estimated or projected. Investors are cautioned not to put undue reliance on forward-looking statements. A further list and description of risks, uncertainties and other matters can be found in our Annual Report on Form 10-K for the fiscal year ended December 31, 2025 and in our subsequent reports on Form 10-Q. Except as required by law, we do not undertake any obligation to publicly update any forward-looking statements whether as a result of any new information, future events, changed circumstances or otherwise.

Biogen Digital Media Disclosure

From time to time, we have used, or expect in the future to use, our investor relations website (investors.biogen.com), the Biogen LinkedIn account ([linkedin.com/company/biogen-](https://www.linkedin.com/company/biogen/)) and the Biogen X account (<https://x.com/biogen>) as a means of disclosing information to the public in a broad, non-exclusionary manner, including for purposes of the SEC's Regulation Fair Disclosure (Reg FD). Accordingly, investors should monitor our investor relations website and these social media channels in addition to our press releases, SEC filings, public conference calls and websites, as the information posted on them could be material to investors.

References:

1. Beniczky, S. et al. Updated classification of epileptic seizures: Position paper of the International League Against Epilepsy. *Epilepsia*. 2025; 1804–1823.
2. Shmueli, S. et al. Mortality in Dravet syndrome: A review, *Epilepsy & Behavior*. 2016: (Pt A) 69-74.
3. Symonds, J. et al. Early childhood epilepsies: epidemiology, classification, aetiology, and socio-economic determinants. *Brain*. 2021;144(9):2879-2891.
4. 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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