Stoke Therapeutics and Biogen Announce Presentations of Long-Term Clinical Data for Zorevunersen, an Investigational Medicine for the Treatment of Dravet Syndrome, at the 2026 American Epilepsy Society Annual Meeting

November 23, 2026

–Phase 1/2a and 4-year open-label extension data support the disease-modifying potential of zorevunersen–

–Results show the ongoing effects of treatment with zorevunersen across multiple measures of disease burden, including seizure frequency, neurodevelopmental outcomes and quality of life–

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

–Phase 3 EMPEROR study is ongoing with data anticipated in Q3 2027–

BEDFORD, Mass. and CAMBRIDGE, Mass., Nov. 23, 2026 (GLOBE NEWSWIRE) -- 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 long-term clinical data from studies of zorevunersen at the 2026 American Epilepsy Society (AES) Annual Meeting, taking place December 4-8 in Denver, Colorado.

Zorevunersen is an investigational antisense oligonucleotide currently being evaluated as a potential first-in-class medicine for the treatment of Dravet syndrome, a severe genetic developmental and epileptic encephalopathy (DEE) characterized by recurrent seizures and significant cognitive and behavioral impairments. Data to be presented at AES support the disease-modifying potential of zorevunersen and represent more than 5 years of clinical experience, spanning ~1 year of treatment in the Phase 1/2a studies and ~4 years of treatment in the open-label extension studies (OLEs). Results show the ongoing effects of treatment with zorevunersen across multiple measures of disease burden, including seizure frequency, neurodevelopmental outcomes and quality of life. Zorevunersen continues to be generally well tolerated, with some patients treated for more than 5 years.

The global, pivotal Phase 3 EMPEROR study is underway to further evaluate the safety and efficacy of zorevunersen. A data readout is anticipated in the third quarter of 2027 to support a planned U.S. New Drug Application (NDA) submission to the U.S. Food and Drug Administration (FDA) in the second half of 2027.

"We are encouraged by the growing awareness of Dravet syndrome within the broader epilepsy community and the deepening appreciation for how a disease-modifying medicine may change the way this disease is treated," said Barry Ticho, M.D., Ph.D., Chief Medical Officer of Stoke Therapeutics. "We look forward to sharing our five years of safety and efficacy data for zorevunersen with the world's leading epilepsy experts at AES as the field prepares for a potential new class of medicines that may bring people with Dravet syndrome the possibility of a different future."

"The Dravet community has waited decades for a medicine that targets the underlying cause of the disease, with the potential to address both the developmental and epileptic manifestations," said Stephanie Fradette, Pharm.D., Head of the Rare Neurology Development Unit at Biogen. "As the Phase 3 EMPEROR study progresses, additional data from the ongoing open-label extension studies further our confidence in zorevunersen's long-term safety and its potential to change the lives of patients and their families."

Stoke Presence at AES 2026

Data Presentations:

Title: Long-term findings with zorevunersen in Dravet syndrome: results through 4 years of treatment in open-label extension studies
Poster Presentation Date & Time: Monday, December 7, 12:00-1:45 PM MST
Poster Presenter: Kelly Knupp, M.D., MSCS, Professor of Pediatrics and Neurology at the University of Colorado Anschutz and the Dravet Program Director and Epilepsy Program Lead at Children's Hospital Colorado
Poster Number: 3.295

Title: Seizure burden outcomes in Dravet syndrome with zorevunersen treatment in Phase 1/2a open-label extension studies
Poster Presentation Date & Time: Monday, December 7, 12:00-1:45 PM MST
Poster Presenter: Linda C. Laux, M.D., Associate Division Head of Neurology, Section Head of the Epilepsy Center, and Lorna S. and James P. Langdon Chair in Pediatric Neurology at Ann & Robert H. Lurie Children's Hospital of Chicago
Poster Number: 3.300

Exhibit Booth #1807: Attendees can learn more about the science behind Dravet syndrome and its impact on patients and families through interactive content and updates from Stoke's *Unseen* disease education campaign. Members of Stoke's Medical Team will also be available to answer questions about the company's ongoing clinical trials.

Scientific Exhibit: Provides an opportunity to examine the evolving understanding of Dravet syndrome, explore outcome measures relevant to potential disease modification, and learn about the zorevunersen clinical development program. During the exhibit, Dravet syndrome expert and zorevunersen clinical trial investigator Professor Andreas Brunklaus (University of Glasgow) will discuss recent clinical data with attendees.

Title: *From Scientific Insights Toward Clinical Advancement in Dravet Syndrome: Updates from the Investigational Zorevunersen Development Program*
Date & Time: Sunday, December 6, 11:00 AM-5:00 PM MST
Location: Bluebird Room 2A

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, sleep problems, 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 (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 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. 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. 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 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, LinkedIn, X, YouTube.

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 design, timing and expected progress of clinical trials, data readouts, regulatory submissions, regulatory decisions and other presentations; and the participation of scientists associated with Stoke making presentations at AES and the presentation of data at AES. 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-) 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. 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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