Stoke Therapeutics and Biogen Present New Data at the 54th Child Neurology Society (CNS) Annual Meeting that Support the Potential of Zorevunersen as a Disease-Modifying Medicine for Dravet Syndrome

October 9, 2025

– An analysis designed to evaluate the potential effects of the Phase 3 zorevunersen dosing regimen showed continuing improvements in cognition and behavior at 2 years; results contrast with minimal change in natural history –

– Improvements in overall clinical status at 3 years in the open-label extension studies reported by clinicians and caregivers in 95% of patients treated with zorevunersen –

BEDFORD, Mass., & CAMBRIDGE, Mass.--(BUSINESS WIRE)--Oct. 9, 2025-- 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 the presentation of longer-term follow-up analyses from the ongoing open-label extension (OLE) studies of zorevunersen that support the potential of zorevunersen as a disease-modifying medicine for Dravet syndrome. These new results were presented at the 54th Child Neurology Society (CNS) Annual Meeting.

New two-year data from an analysis that was initially performed to understand the potential effects of the Phase 3 dosing regimen on cognition and behavior showed continuing improvements at two years. These results contrast with findings from a two-year natural history study in which patients with Dravet syndrome who were treated with standard of care showed minimal changes in cognition and behavior. In addition, the companies presented three-year results from analyses of Clinical and Caregiver Global Impression of Change (CGI-C and CaGI-C) scales measuring overall clinical status, which complement previously presented EQ-VAS caregiver-reported quality of life improvements. Caregivers and clinicians separately reported similar improvements in overall clinical status in 95% of patients treated with zorevunersen (n=19).

"Data from the ongoing open-label extension studies are helping to shape our understanding of the long-term effects of zorevunersen on seizures, cognition, behavior and overall functioning, which together support the potential for disease modification," said Kelly Knupp, MD, 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. "The natural history data continue to provide important context for the effects we are seeing in patients treated with zorevunersen. Improvements in cognition and behavior are not something we see in patients with Dravet syndrome, and rarely do we see such strong correlation between caregiver and clinician-reported outcomes. Together, these data are starting to paint a fuller picture of the potential impact disease modification could have on a patient's overall health and daily living."

These data were among several analyses included in a poster presentation of data from the Phase 1/2a and ongoing OLE studies of zorevunersen. Safety and tolerability were the primary endpoints of these studies. In addition, effects on major motor seizure frequency, cognition and behavior were assessed as secondary endpoints. As previously reported, results showed substantial and durable reductions in major motor seizure frequency and improvements in multiple measures of cognition and behavior on top of a background of standard anti-seizure medicines through three years. The most substantial reductions were observed among patients who were treated with loading doses of 70mg in the Phase 1/2a study followed by maintenance doses of 45mg. This regimen is now being evaluated in the Phase 3 EMPEROR study.

Summary of Safety Data

Eighty-one patients received at least one dose of zorevunersen and have been evaluated for safety. Zorevunersen has been generally well tolerated across the Phase 1/2a and OLE studies. Study drug related treatment emergent adverse events (TEAEs) were observed in 30% (24/81) and 53% (40/75) of patients treated in the Phase 1/2a and OLE studies, respectively. The most common study drug related TEAE was CSF protein elevations reported in 14% (11/81) of patients in the Phase 1/2a studies and 44% (33/75) of patients in the OLE studies. CSF protein elevations (>50 mg/dL) occurred in 42% (34/81) of patients in the Phase 1/2a studies and 86% (62/72) of patients in the OLE studies. No related clinical manifestations have been observed although one patient discontinued treatment due to elevated CSF protein levels. Treatment-emergent serious adverse events (TESAEs) were reported in 22% (18/81) and 29% (22/75) of patients in the Phase 1/2a and OLE studies, respectively, all of which were assessed to be unrelated to study drug except one patient who experienced SUSARs.

Details of the Presentation

Title: Zorevunersen demonstrates disease-modifying potential in patients with Dravet syndrome through durable seizure reduction and continuing improvements in cognition, behavior, and functioning through 36 months of treatment in open-label extension studies
Presenter: Joseph Sullivan, M.D., FAES, Professor of Neurology and Pediatrics and Director of the Pediatric Epilepsy Center of Excellence at the University of California San Francisco
Session: Poster Review and Guided Abstract Tours
Date and Time: Thursday, October 9, 12:30-1:45 PM and 5:30-7:00 PM ET
Poster Number and Location: Poster #92, Hall C, Charlotte Convention Center, Charlotte, NC

The presentation will be available for download on the Stoke Therapeutics website under the Investors & News tab.

About Dravet Syndrome

Dravet syndrome is a severe developmental and epileptic encephalopathy (DEE) characterized by severe, 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. More than 90 percent of patients continue to experience seizures despite treatment with the best available anti-seizure medicines. 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. 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.[1] 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 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/.

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; and the design, timing and results of the Phase 3 EMPEROR study. Statements including words such as "anticipate," "could," "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 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 Stoke's collaborators were to breach or terminate their agreements, it 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 through mid-2028; and the other risks and uncertainties described under the heading "Risk Factors" in Stoke's 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 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 potential benefits, safety and efficacy of zorevunersen; potential regulatory discussions, submissions and approvals and the timing thereof; the treatment of Dravet syndrome; the anticipated benefits, risks and potential of Biogen's collaboration arrangements with Stoke; 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.

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, 2024 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.

Reference:

1. 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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Stoke Media & Investor Contacts:
Dawn Kalmar
Chief Communications Officer
dkalmar@stoketherapeutics.com
781-303-8302

Doug Snow
Director, Communications & Investor Relations
IR@stoketherapeutics.com
508-642-6485

Biogen Media Contact:
Madeleine Shin
Public.affairs@biogen.com
+ 1 781 464 3260

Biogen Investor Contact:
Tim Power
IR@biogen.com
+1 781 464 2442

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