



## Stoke Therapeutics and Biogen Present Data at the 36th International Epilepsy Congress that Support the Potential for Zorevunersen to be the First Disease-Modifying Medicine for Dravet Syndrome

September 2, 2025

- Durable reductions in seizures and continuing improvements in cognition and behavior through 3 years in patients who continued to receive zorevunersen in the open-label extension studies –
- Substantial increase in seizure-free days and continuous improvements in quality of life demonstrated in patients already taking standard of care anti-seizure medicines –
- Zorevunersen generally well tolerated across all studies –
- Data support pivotal Phase 3 EMPEROR study now underway –

BEDFORD, Mass. & CAMBRIDGE, Mass.--(BUSINESS WIRE)--Sep. 2, 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 data from Phase 1/2a and open-label extension (OLE) studies of zorevunersen that support the potential for zorevunersen to be the first disease-modifying medicine for Dravet syndrome. Findings were presented at the 36<sup>th</sup> International Epilepsy Congress (IEC) in Lisbon, Portugal. Data support the EMPEROR Phase 3 study of zorevunersen that is now underway to further evaluate this investigational antisense oligonucleotide.

"The burden of Dravet syndrome starts with seizures, but natural history studies make clear the far-reaching and lifelong impacts this disease has on nearly every aspect of daily living for patients and their families," said Professor Andreas Brunklaus, Consultant Paediatric Neurologist at the Royal Hospital for Children in Glasgow, Honorary Professor at the University of Glasgow, and a zorevunersen study investigator. "The durable reductions in seizures and improvements in cognition, behavior and quality of life demonstrated in the first year of treatment, again in the second year and now into the third year, along with a generally well tolerated safety profile, give us confidence in the disease modifying potential of zorevunersen."

Data presented from the Phase 1/2a and open-label extension studies represent more than four years of clinical experience with zorevunersen. Across the studies, substantial and durable reductions in major motor seizure frequency and continuing improvements in cognition, behavior and quality of life were shown on top of standard anti-seizure regimens. More than 700 doses of zorevunersen have been administered to date.

### Efficacy Results From the Initial Treatment Period of the Phase 1/2a Studies

The Phase 1/2a studies evaluated single and multiple doses of zorevunersen up to 70mg with a primary endpoint of safety. Effects on major motor seizure frequency were evaluated as a secondary endpoint. A dose relationship was demonstrated among patients treated with multiple doses of zorevunersen (30mg, 45mg, 70mg). Patients treated with an initial 2 or 3 doses of 70mg (n=10) experienced the most substantial reductions in seizures. A median reduction in seizures of 84.8% and a median increase of eight seizure-free days per 28 days were observed at 3 months after the last dose. Patients who received initial 70mg doses of zorevunersen in the Phase 1/2a studies also showed the most substantial improvements in quality-of-life outcomes as measured by the EuroQol Visual Analog Scale (EQ-VAS), a component of the Euro-QoL-5D Youth.

### Efficacy Results From the Continuing Treatment Period in the OLEs Through 3 Years

Following treatment in the Phase 1/2a studies, 94% (75/80) of eligible patients continued treatment in the OLEs. Through three years, 77% (58/75) of these patients remain in the studies. Overall, reductions in major motor seizure frequency were sustained through three years of treatment in the OLE studies. The most substantial reductions in seizure frequency were observed among patients treated with initial doses of 70mg in the Phase 1/2a studies.

Standard assessments are used in the OLE studies to evaluate neurodevelopment, functioning, clinical status and quality of life for all patients. Vineland Adaptive Behavior Scales, Third Edition (Vineland-3) was used to measure changes in communication, motor skills, socialization and daily living. Subdomains of Vineland-3 were measured using raw scores and compared to each patient's baseline at OLE entry. Continuing improvements were demonstrated among patients who received zorevunersen every four months in the OLEs. Through three years, data indicated improvements of 4.3-9.7 raw score points across eight key subdomains, including 7.6 points in expressive communication and 6.1 points in receptive communication. Caregivers have identified a 1-3 point change in raw scores for Vineland-3 subscales as meaningful. Patients also experienced ongoing improvements in quality of life as measured by EQ-VAS, with an 18-point improvement demonstrated through three years. EQ-VAS is a validated visual analogue scale ranging from 0 to 100 (worst to best imaginable health).

### 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.

All presentations are available for download on the Stoke Therapeutics website under the Investors & News tab.

### **About the Phase 1/2a and Open-Label Extension Studies of Zorevunersen**

Two Phase 1/2a open-label, multicenter studies were conducted and 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 convulsive seizure frequency, overall clinical status 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 convulsive 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. The OLE studies are ongoing.

### **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.<sup>1</sup>

### **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. Study 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. An open-label extension treatment period will allow all patients the opportunity to receive treatment with zorevunersen following the 52-week treatment period. 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), EuroQol Visual Analog Scale (EQ-VAS) and the Bayley Scales of Infant Development (BSID-IV). EMPEROR has initiated in the United States, United Kingdom, Japan and is planned for Europe. For more information on the EMPEROR study, please visit <https://www.emperorstudy.com/> and <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/>.

### **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](http://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 clinical trials, data readouts, regulatory decisions and other presentations for zorevunersen. 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 to 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 and for patients of Dravet syndrome; the potential benefits, safety and efficacy of zorevunersen and continued treatment with 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 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](http://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, 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](#).

**Stoke Media & Investor Contacts:**

Dawn Kalmar  
Chief Communications Officer  
[dkalmar@stoketherapeutics.com](mailto:dkalmar@stoketherapeutics.com)  
781-303-8302

Doug Snow  
Director, Communications & Investor Relations  
[IR@stoketherapeutics.com](mailto:IR@stoketherapeutics.com)  
508-642-6485

**Biogen Media Contact:**

Madeleine Shin  
[Public.affairs@biogen.com](mailto:Public.affairs@biogen.com)  
+ 1 781 464 3260

**Biogen Investor Contact:**

Tim Power  
[IR@biogen.com](mailto:IR@biogen.com)  
+1 781 464 2442

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