

UNITED STATES
SECURITIES AND EXCHANGE COMMISSION
WASHINGTON, D.C. 20549

FORM 8-K

CURRENT REPORT
Pursuant to Section 13 or 15(d)
of the Securities Exchange Act of 1934

Date of Report (Date of earliest event reported): December 4, 2024

Stoke Therapeutics, Inc.

(Exact name of Registrant as Specified in Its Charter)

Delaware
(State or Other Jurisdiction
of Incorporation)

001-38938
(Commission
File Number)

47-1144582
(IRS Employer
Identification No.)

45 Wiggins Ave
Bedford, Massachusetts
(Address of Principal Executive Offices)

01730
(Zip Code)

Registrant's Telephone Number, Including Area Code: (781) 430-8200

Not Applicable
(Former Name or Former Address, if Changed Since Last Report)

Check the appropriate box below if the Form 8-K filing is intended to simultaneously satisfy the filing obligation of the registrant under any of the following provisions:

- Written communications pursuant to Rule 425 under the Securities Act (17 CFR 230.425)
- Soliciting material pursuant to Rule 14a-12 under the Exchange Act (17 CFR 240.14a-12)
- Pre-commencement communications pursuant to Rule 14d-2(b) under the Exchange Act (17 CFR 240.14d-2(b))
- Pre-commencement communications pursuant to Rule 13e-4(c) under the Exchange Act (17 CFR 240.13e-4(c))

Securities registered pursuant to Section 12(b) of the Act:

Title of each class	Trading Symbol(s)	Name of each exchange on which registered
Common Stock, \$0.0001 par value per share	STOK	Nasdaq Global Select Market

Indicate by check mark whether the registrant is an emerging growth company as defined in Rule 405 of the Securities Act of 1933 (§ 230.405 of this chapter) or Rule 12b-2 of the Securities Exchange Act of 1934 (§ 240.12b-2 of this chapter).

Emerging growth company

If an emerging growth company, indicate by check mark if the registrant has elected not to use the extended transition period for complying with any new or revised financial accounting standards provided pursuant to Section 13(a) of the Exchange Act.

Item 7.01. Regulation FD.

On December 4, 2024, Stoke Therapeutics, Inc. (the “Company”) issued a press release (the “BTD Press Release”) announcing that it received Breakthrough Therapy Designation from the U.S. Food & Drug Administration (the “FDA”) for zorevunersen, a product candidate for the treatment of Dravet syndrome, a severe and progressive genetic epilepsy. A copy of the BTD Press Release is furnished as Exhibit 99.1 to this Current Report on Form 8-K and incorporated by reference herein.

On December 6, 2024, the Company issued a press release (the “OLE Press Release”) announcing new data from an analysis of nine patients treated with an initial 2 or 3 doses of 70mg, followed by 45mg maintenance dosing in the Phase 1/2a and open-label extension (OLE) studies of zorevunersen. A copy of the OLE Press Release is furnished as Exhibit 99.2 to this Current Report on Form 8-K and incorporated by reference herein.

The information furnished under this Item 7.01, including Exhibits 99.1 and 99.2, shall not be deemed “filed” for purposes of Section 18 of the Securities Exchange Act of 1934, as amended (the “Exchange Act”), or otherwise subject to the liabilities of that section, nor shall it be deemed incorporated by reference into any other filing under the Exchange Act or the Securities Act of 1933, as amended, except as expressly set forth by specific reference in such a filing.

Item 8.01 Other Events.

As noted in the BTD Press Release, the Company received Breakthrough Therapy Designation from the FDA for zorevunersen for the treatment of Dravet syndrome with a confirmed mutation, not associated with gain-of-function, in the *SCN1A* gene.

The OLE Press Release provides a business update with respect certain key findings from the OLE studies of zorevunersen, including the following:

Substantial and Durable Reductions in Convulsive Seizure Frequency

- Previously reported end-of-Phase 1/2a study data from patients treated with two or three doses of 70mg of zorevunersen showed substantial and sustained reductions in convulsive seizure frequency of 85% at 3 months (n=10) and 74% at six months (n=9) post-last dose.
- The nine patients who continued treatment with at least two doses of 45mg of zorevunersen in the OLE study sustained at least a 50% median reduction from baseline at each month of the OLE and demonstrated an 87% median reduction at month eight, the latest timepoint for which data have been assessed for these patients.

Continuous Improvements in Multiple Measures of Cognition and Behavior

- Patients experienced continuous improvements in multiple measures of cognition and behavior as measured by the Vineland-3 through 2 years of treatment with ongoing maintenance dosing in the OLEs. Additional improvements were indicated within the first nine months of treatment among patients in the Phase 1/2a ADMIRAL study.

Item 9.01 Financial Statements and Exhibits.

(d) Exhibits

Exhibit Number	Description
99.1	Press Release dated December 4, 2024
99.2	Press Release dated December 6, 2024
104	Cover Page Interactive Data File (the cover page XBRL tags are embedded within the inline XBRL document).

Forward-Looking Statements

This Current Report on Form 8-K contains forward-looking statements within the meaning of the U.S. Private Securities Litigation Reform Act of 1995, including, but not limited to: the advantages that may be achieved by TANGO; the ability of zorevunersen (STK-001) to treat the underlying causes of Dravet syndrome and reduce seizures or show improvements in behavior or cognition at the indicated dosing levels or at all; the timing and expected progress of clinical trials, data readouts, regulatory decisions and other presentations for zorevunersen; the potential for zorevunersen to be the first disease-modifying therapy for Dravet syndrome; the timing of regulatory interactions or the outcomes thereof; the Company's expectations, plans, aspirations and goals, including those related to the potential of zorevunersen. Statements including words such as "anticipate," "believe," "hope," "plan," "will," "continue," "expect," "ongoing," or "potential" 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 of, and ultimately commercialize its product candidates, including zorevunersen; the timing of data readouts and interim and final results of preclinical and clinical trials; preclinical and clinical data are voluminous and detailed, and regulatory authorities may interpret or weigh the importance of data differently and reach different conclusions than us or others, request additional information, have additional recommendations or change their guidance or requirements before or after approval; receiving Breakthrough Therapy Designation may not lead to a faster development or regulatory review or approval and does not mean zorevunersen will receive marketing approval; the Company's ability to fund development activities and achieve development goals; the Company's ability to protect its intellectual property; global business, political and macroeconomic conditions, including inflation, interest rate volatility, cybersecurity events, uncertainty with respect to the federal budget, instability in the global banking system and volatile market conditions, and global events, including public health crises and ongoing geopolitical conflicts, such as the conflicts in Ukraine and the Middle East; and other risks and uncertainties described under the heading "Risk Factors" in the Company's Annual Report on Form 10-K for the year ended December 31, 2023, its quarterly reports on Form 10-Q and the other documentation it files from time to time with the Securities and Exchange Commission. These forward-looking statements speak only as of the date of this press release, and the Company undertakes no obligation to revise or update any forward-looking statements to reflect events or circumstances after the date hereof.

SIGNATURE

Pursuant to the requirements of the Securities Exchange Act of 1934, the registrant has duly caused this report to be signed on its behalf by the undersigned hereunto duly authorized.

STOKE THERAPEUTICS, INC.

Date: December 6, 2024

By: /s/ Thomas E. Leggett

Thomas E. Leggett
Chief Financial Officer



Stoke Therapeutics Receives FDA Breakthrough Therapy Designation for Zorevunersen for the Treatment of Dravet Syndrome

- Supported by evidence from clinical studies that indicate that zorevunersen may demonstrate substantial improvement over available therapies –*
- Update on the company's plans for a global, randomized, controlled Phase 3 registrational study anticipated by year-end –*

BEDFORD, MA, December 4, 2024 – Stoke Therapeutics, Inc. (Nasdaq: STOK), a biotechnology company dedicated to restoring protein expression by harnessing the body's potential with RNA medicine, today announced that it has received Breakthrough Therapy Designation from the U.S. Food and Drug Administration (FDA) for zorevunersen for the treatment of Dravet syndrome with a confirmed mutation, not associated with gain-of-function, in the *SCN1A* gene. Zorevunersen is being developed as potentially the first disease-modifying medicine for the treatment of Dravet syndrome.

Clinical data from the Phase 1/2a and open-label extension (OLE) studies of zorevunersen demonstrated substantial and sustained reductions in seizure frequency and continuous improvements in multiple measures of cognition and behavior. These effects were observed on top of the best available anti-seizure medicines, the current standard of care. Zorevunersen was generally well tolerated across the studies. To date, more than 600 doses of zorevunersen have been administered to patients, some of whom have been on treatment for more than three years.

Discussions with the FDA and other global regulatory agencies regarding a global, randomized, controlled Phase 3 registrational study of zorevunersen continue to progress. The Company plans to provide an update on its Phase 3 registrational plans by the end of the year.

"The FDA's Breakthrough Therapy designation for zorevunersen is supported by promising clinical data that suggest that zorevunersen has the potential to demonstrate substantial improvement over current treatments for Dravet syndrome," said Shamim Ruff, Chief Regulatory Affairs Officer, Stoke Therapeutics. "By helping the body restore naturally occurring NaV1.1 protein levels, zorevunersen is designed to treat the underlying cause of the disease. We thank the FDA for their support and look forward to continuing to work together closely to efficiently advance zorevunersen into a registrational Phase 3 study."

"This designation brings new hope to the many patients with Dravet syndrome who continue to experience treatment-resistant seizures and a myriad of health and quality of life complications despite the availability of symptomatic treatments," said Mary Anne Meskis, Executive Director, Dravet Syndrome Foundation. "Our organization has been engaging with the FDA to ensure greater awareness and understanding of Dravet syndrome. We are encouraged by the Agency's shared sense of urgency for the development of innovative new medicines that could help address the gaps left by current treatments by treating the underlying cause of the disease."

Breakthrough Therapy designation is a process designed to expedite the development and review of drugs that are intended to treat a serious condition and preliminary clinical evidence indicates that the drug may demonstrate substantial improvement over available therapy on a clinically-significant endpoint(s). This designation grants zorevunersen access to all Fast Track designation features, intensive guidance on an efficient drug development program and an organizational commitment involving senior FDA managers.

About Dravet Syndrome

Dravet syndrome is a severe and progressive genetic epilepsy characterized by frequent, prolonged and refractory seizures, beginning within the first year of life. Dravet syndrome is difficult to treat and has a poor long-term prognosis. Complications of the disease often contribute to a poor quality of life for patients and their caregivers. The effects of the disease go beyond seizures and 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. The disease is classified as a developmental and epileptic encephalopathy due to the developmental delays and cognitive impairment associated with the disease. Compared with the general epilepsy population, people living with Dravet syndrome have a higher risk of sudden unexpected death in epilepsy, or SUDEP. There are no approved disease-modifying therapies for people living with Dravet syndrome. One out of 16,000 babies are born with Dravet syndrome, which is not concentrated in a particular geographic area or ethnic group.

About Zorevunersen (STK-001)

Zorevunersen is an investigational new medicine for the treatment of Dravet syndrome currently being evaluated in ongoing clinical trials. Stoke believes that zorevunersen, a proprietary antisense oligonucleotide (ASO), has the potential to be the first disease-modifying therapy to address the genetic cause of Dravet syndrome. Zorevunersen is designed to upregulate NaV1.1 protein expression by leveraging the non-mutant (wild-type) copy of the *SCN1A* gene to restore physiological NaV1.1 levels, thereby reducing both occurrence of seizures and significant non-seizure comorbidities. Zorevunersen has been granted orphan drug designation by the FDA and the EMA, and rare pediatric disease designation by the FDA as a potential new treatment for Dravet syndrome.

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 protein levels. Stoke's first compound, zorevunersen, is in clinical testing for the treatment of Dravet syndrome, a severe and progressive genetic epilepsy. Dravet syndrome is one of many diseases caused by a

haploinsufficiency, in which a loss of ~50% of normal protein levels leads to disease. Stoke is pursuing the development of STK-002 for the treatment of autosomal dominant optic atrophy (ADOA), the most common inherited optic nerve disorder. Stoke's initial focus is haploinsufficiencies and diseases of the central nervous system and the eye, although proof of concept has been demonstrated in other organs, tissues, and systems, supporting its belief in the broad potential for its proprietary approach. Stoke is headquartered in Bedford, Massachusetts with offices in Cambridge, Massachusetts. For more information, visit <https://www.stoketherapeutics.com/>.

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 timing and expected progress of clinical trials, data readouts, regulatory meetings, regulatory decisions and other presentations. Statements including words such as "expect," "plan," "will," "continue," or "ongoing" 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 our 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 of, and ultimately commercialize its product candidates, including zorevunersen; fast track or breakthrough designations by the FDA may not lead to faster development or regulatory review or approval process and do not increase the likelihood that zorevunersen will receive marketing approval; the timing of data readouts and interim and final results of preclinical and clinical trials; the receipt and timing of potential regulatory decisions; 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; the Company's ability to fund development activities and achieve development goals, including expectations regarding its collaboration with Acadia Pharmaceuticals; the Company's ability to protect its intellectual property; the direct or indirect impact of global business, political and macroeconomic conditions, including inflation, interest rate volatility, cybersecurity events, uncertainty with respect to the federal budget, instability in the global banking system and volatile market conditions, and global events, including public health crises, and ongoing geopolitical conflicts, such as the conflicts in Ukraine and the Middle East; and other risks and uncertainties described under the heading "Risk Factors" in the Company's Annual Report on Form 10-K for the year ended December 31, 2023, its quarterly reports on Form 10-Q, and the other documents it files from time to time with the Securities and Exchange Commission. These forward-looking statements speak only as of the date of this press release, and the Company undertakes no obligation to revise or update any forward-looking statements to reflect events or circumstances after the date hereof.

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

Stoke Therapeutics Presents New Open-Label Extension (OLE) Study Data That Further Support the Potential for Zorevunersen as a Disease-Modifying Medicine for the Treatment of Dravet Syndrome

- Substantial and durable reductions in convulsive seizure frequency observed in patients treated with 2 or 3 doses of 70mg followed by 45mg maintenance dosing on top of the best available anti-seizure medicines –*
- Patients experienced continuous improvements in multiple measures of cognition and behavior with ongoing treatment through 2 years –*
- Data support proposed Phase 3 registrational study regimen; Update anticipated before year-end –*
- Zorevunersen generally well-tolerated across the studies –*
- Data to be presented at the American Epilepsy Society (AES) 2024 Annual Meeting; Company to host virtual event for investors and analysts on Monday, December 9 at 8:30 am Eastern (5:30 am Pacific) –*

BEDFORD, Mass., December 6, 2024 – Stoke Therapeutics, Inc. (Nasdaq: STOK), a biotechnology company dedicated to restoring protein expression by harnessing the body's potential with RNA medicine, today announced new data from an analysis of nine patients treated with an initial 2 or 3 doses of 70mg, followed by 45mg maintenance dosing in the Phase 1/2a and open-label extension (OLE) studies of zorevunersen. Substantial and durable reductions in convulsive seizure frequency were observed in these patients who received zorevunersen on top of the best available anti-seizure medicines. In addition, patients treated in the OLE studies showed continuous improvements in multiple measures of cognition and behavior with ongoing treatment through 2 years. Zorevunersen was generally well tolerated across the studies. Together, these data support the company's proposed Phase 3 registrational study regimen and its efforts to develop zorevunersen as a disease-modifying medicine for the treatment of Dravet syndrome.

The company will host a virtual educational event for investors and research analysts on Monday, December 9 at 8:30 am Eastern (5:30 am Pacific).

“Dravet syndrome is stressful to live with and challenging to treat. Anti-seizure medicines are helpful in reducing seizures but can only do so much for patients who continue to experience significant and life-altering limitations in many aspects of neurodevelopment and daily living,” said 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. “The substantial and durable reductions in seizures, as well as the continuous gains in multiple measures of behavior and cognition through 2 years in patients treated in these studies have never been seen before in studies of Dravet syndrome. I am encouraged by these data and convinced that we are on the verge of a new era in the treatment of this disease.”

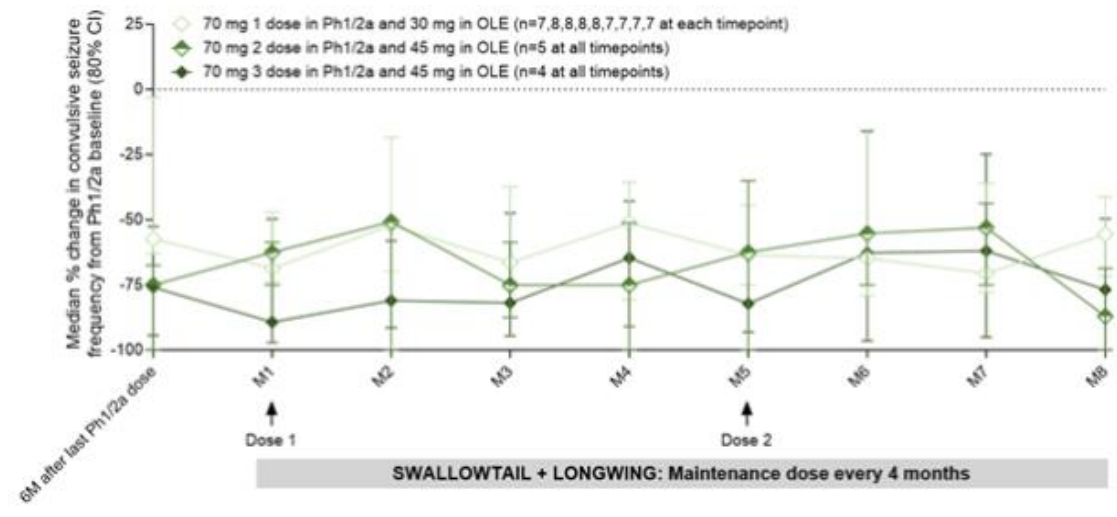
“The new long-term data give us a more complete and convincing picture of the potential for zorevunersen as a disease-modifying medicine,” said Barry Ticho, M.D., Ph.D., Chief Medical Officer of Stoke Therapeutics. “Patients entered our studies with high seizure rates despite being treated with the best available anti-seizure medicines. The durability of the substantial reductions in their seizures, particularly among those treated with initial doses of 70mg are remarkable and support a highly differentiated mechanism of action for zorevunersen. These data, combined with the continuous improvements in cognition and behavior, are highly supportive of our plans to conduct a Phase 3 study and the dose regimen currently under discussion with global regulatory agencies.”

Key Study Finding: Substantial and Durable Reductions in Convulsive Seizure Frequency Poster 2.379 and 2.364

Previously reported end-of-Phase 1/2a study data from patients treated with two or three doses of 70mg of zorevunersen showed substantial and sustained reductions in convulsive seizure frequency of 85% at 3 months (n=10) and 74% at six months (n=9) post-last dose.

The Company is now reporting data for the nine patients who continued treatment with at least two doses of 45mg of zorevunersen in the OLE study. These patients sustained at least a 50% median reduction from baseline at each month of the OLE and demonstrated an 87% median reduction at month eight, the latest timepoint for which data have been assessed for these nine patients. See Figure 1.

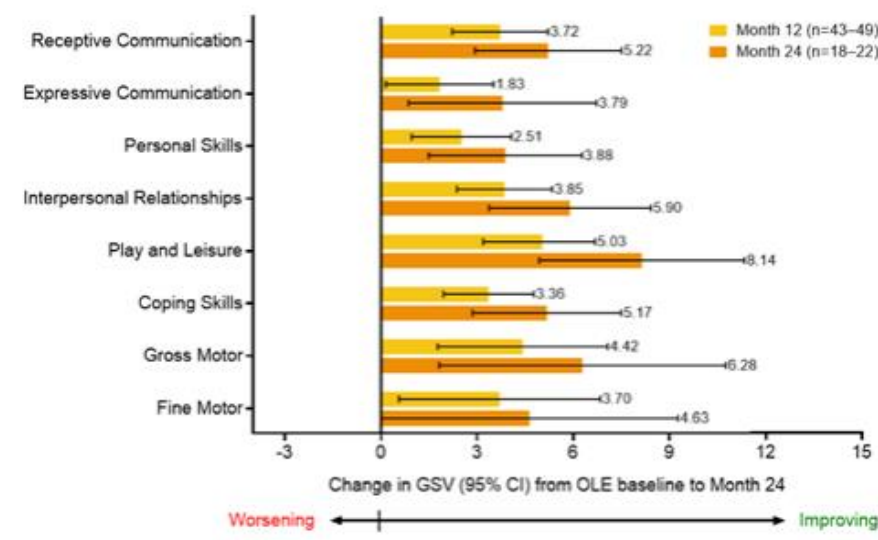
Figure 1. Substantial and Durable Reductions in Convulsive Seizure Frequency from Baseline in Patients who Received 70mg zorevunersen in Phase 1/2a



Key Study Finding: Continuous Improvements in Multiple Measures of Cognition and Behavior

Patients experienced continuous improvements in multiple measures of cognition and behavior as measured by the Vineland-3 through 2 years of treatment with ongoing maintenance dosing in the OLEs. Additional improvements were indicated within the first nine months of treatment among patients in the Phase 1/2a ADMIRAL study. See Figure 2.

Figure 2. Continuing Improvements in Vineland-3 Subdomain Growth Scale Values from OLE Baseline



Key Safety Findings

At the time of the analysis, 81 patients had been treated with zorevunersen in the Phase 1/2a studies. Seventy-four patients who completed the Phase 1/2a studies and were eligible enrolled in the OLEs. As of June 2024, 82% (61/74) remained in the OLE studies. Safety findings from the studies are consistent with prior disclosures and are summarized below.

- Zorevunersen was generally well-tolerated across the Phase 1/2a and OLE studies.
- In the Phase 1/2a studies:
 - 30% (24/81) of patients experienced a treatment-emergent adverse event (TEAE) that was related to study drug. The most common were CSF protein elevations and procedural vomiting; and
 - 22% (18/81) of patients had a treatment-emergent serious adverse event. These events were assessed as unrelated to study drug except for the previously reported case of one patient who experienced Suspected Unexpected Serious Adverse Reactions (SUSARs).
- A greater incidence of CSF protein elevation was observed in the OLEs. 79% (56/71) of patients in the OLEs had at least 1 CSF protein value >50 mg/dL. No clinical manifestations have been observed in these patients.
- Across the studies, one patient discontinued treatment due to study drug. As previously reported, this patient discontinued treatment in the OLE due to elevated CSF protein.

Additional Presentations

Small Changes on the Vineland-3 are Meaningful to Caregivers of Patients with Dravet Syndrome

Poster: 3.383

The Company will also present the results of a study of caregivers and clinical experts that was designed to evaluate what constitutes meaningful change on the Vineland-3 and to generate insight into caregiver perceptions of the key signs, symptoms and impacts of Dravet syndrome. The study found that small changes in adaptive behavior, as measured by the Vineland-3, are considered clinically meaningful by both caregivers of patients with Dravet syndrome and clinical experts. Changes of 2 to 3 points in growth scale values across subdomains were considered meaningful by at least 50% of caregivers. Consistent with other publications, the Expressive Communication and Receptive Communication subdomains were ranked by caregivers as the most important areas to improve with treatment. The study further defines meaningful change thresholds on the Vineland-3 scale to explore cognitive and behavioral benefits in clinical trials of potential disease-modifying treatments.

Spectral EEG Analysis Demonstrates Decreased Slow-wave Activity in Patients with Dravet Syndrome after Treatment with Zorevunersen, an Antisense Oligonucleotide

Poster: 3.407

An EEG analysis of 74 patients treated in clinical studies of zorevunersen showed a reduction in slow-wave activity following zorevunersen administration. The treatment effect on spectral power is most pronounced at the highest zorevunersen doses, with reductions in slow-wave activity sustained for months after the last dose. Topographical spectral changes indicate that zorevunersen produces a widespread and durable effect across the brain.

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

Stoke Therapeutics Analyst and Investor Virtual Event

Stoke will host a virtual event with discussions led by leading clinicians and patient advocates to offer analysts and investors an opportunity to learn more about Dravet syndrome, its overall effects, and the potential real-world impacts of a disease-modifying medicine. As part of this event, the clinicians are expecting to share anecdotes and visuals from their experience treating patients in the clinical studies of zorevunersen.

Title: Understanding Dravet Syndrome: The Unmet Need and Potential for Disease-Modification

Date and Time: Monday, December 9, 8:30-9:30 AM EST (5:30-6:30 AM PST)

Presenters: Edward M. Kaye, M.D., CEO of Stoke Therapeutics, 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; Andreas Brunklaus M.D., Consultant Paediatric Neurologist at the Royal Hospital for Children, Glasgow, Honorary Professor at the University of Glasgow, member of Dravet Syndrome UK's Medical Advisory Board; Mary Anne Meskis, Executive Director, Dravet Syndrome Foundation; and Veronica Hood, PhD, Scientific Director, Dravet Syndrome Foundation

Webcast Link: <https://edge.media-server.com/mmc/p/bv6h2oxs>

About Dravet Syndrome

Dravet syndrome is a severe and progressive genetic epilepsy characterized by frequent, prolonged and refractory seizures, beginning within the first year of life. Dravet syndrome is difficult to treat and has a poor long-term prognosis. Complications of the disease often contribute to a poor quality of life for patients and their caregivers. The effects of the disease go beyond seizures and 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. The disease is classified as a developmental and epileptic encephalopathy due to the developmental delays and cognitive impairment associated with the disease. Compared with the general epilepsy population, people living with Dravet syndrome have a higher risk of sudden unexpected death in epilepsy, or SUDEP. There are no approved disease-modifying therapies for people living with Dravet syndrome. One out of 16,000 babies are born with Dravet syndrome, which is not concentrated in a particular geographic area or ethnic group.

About Zorevunersen

Zorevunersen is an investigational new medicine for the treatment of Dravet syndrome currently being evaluated in ongoing clinical trials. Stoke believes that zorevunersen, a proprietary antisense oligonucleotide (ASO), has the potential to be the first disease-modifying therapy to address the genetic cause of Dravet syndrome. Zorevunersen is designed to upregulate NaV1.1 protein expression by leveraging the non-mutant (wild-type) copy of the *SCN1A* gene to restore physiological NaV1.1 levels, thereby reducing both occurrence of seizures and significant non-seizure comorbidities. Zorevunersen 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.

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 protein levels. Stoke's first compound, zorevunersen (STK-001), is in clinical testing for the treatment of Dravet syndrome, a severe and progressive genetic epilepsy. Dravet syndrome is one of many diseases caused by a haploinsufficiency, in which a loss of ~50% of normal protein levels leads to disease. Stoke is pursuing the development of STK-002 for the treatment of autosomal dominant optic atrophy (ADOA), the most common inherited optic nerve disorder. Stoke's initial focus is haploinsufficiencies and diseases of the central nervous system and the eye, although proof of concept has been demonstrated in other organs, tissues, and systems, supporting its belief in the broad potential for its proprietary approach. Stoke is headquartered in Bedford, Massachusetts with offices in Cambridge, Massachusetts. For more information, visit <https://www.stoketherapeutics.com/>.

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 (STK-001) to treat the underlying causes of Dravet syndrome and reduce seizures or show improvements in behavior or cognition at the indicated dosing levels or at all; the timing and expected progress of clinical trials, data readouts, regulatory decisions and

other presentations for zorevunersen, including the timing and presentation of data at AES 2024; the potential for zorevunersen to be the first disease-modifying therapy for Dravet syndrome; the timing of regulatory interactions or the outcomes thereof; our expectations, plans, aspirations and goals, including those related to the potential of zorevunersen. Statements including words such as “anticipate,” “believe,” “hope,” “plan,” “will,” “continue,” “expect,” “ongoing,” or “potential” 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 our results to differ materially from those expressed or implied by such forward-looking statements, including, but not limited to, risks and uncertainties related to: our ability to advance, obtain regulatory approval of, and ultimately commercialize our product candidates, including zorevunersen; the timing of data readouts and interim and final results of nonclinical and clinical trials; nonclinical and clinical data are voluminous and detailed, and regulatory authorities may interpret or weigh the importance of data differently and reach different conclusions than us or others, request additional information, have additional recommendations or change their guidance or requirements before or after approval; receiving Breakthrough Therapy Designation may not lead to a faster development or regulatory review or approval and does not mean zorevunersen will receive marketing approval; our ability to fund development activities and achieve development goals; our ability to protect our intellectual property; global business, political and macroeconomic conditions, including inflation, interest rate volatility, cybersecurity events, uncertainty with respect to the federal budget, instability in the global banking system and volatile market conditions, and global events, including public health crises and ongoing geopolitical conflicts, such as the conflicts in Ukraine and the Middle East; and other risks and uncertainties described under the heading “Risk Factors” in our Annual Report on Form 10-K for the year ended December 31, 2023, our quarterly reports on Form 10-Q and the other documentation we file from time to time with the Securities and Exchange Commission. These forward-looking statements speak only as of the date of this presentation, and we undertake no obligation to revise or update any forward-looking statements to reflect events or circumstances after the date hereof.

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