Stoke Therapeutics Announces Plans to Present New Data that Support Zorevunersen as Potentially the First Disease-Modifying Medicine for Dravet Syndrome at the American Epilepsy Society 2024 Annual Meeting

December 2, 2024

– Company to host a virtual educational event for investors and research analysts on Monday, December 9 at 8:30 am Eastern (5:30 am Pacific) –

BEDFORD, Mass.--(BUSINESS WIRE)--Dec. 2, 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 several presentations at the American Epilepsy Society (AES) 2024 Annual Meeting, taking place December 6 – 10, in Los Angeles, California. In addition, the Company will host a virtual event with discussions led by leading clinicians and patient advocates for investors and research analysts on Monday, December 9 at 8:30 am Eastern (5:30 am Pacific). The Company is advancing zorevunersen as potentially the first disease-modifying medicine for the treatment of Dravet syndrome.

"We look forward to presenting positive new data from patients with Dravet syndrome who were already receiving the best available anti-seizure medicines and then were treated with initial doses of 70mg followed by 45mg maintenance dosing of zorevunersen," said Barry Ticho, M.D., Ph.D., Chief Medical Officer of Stoke Therapeutics. "These data underscore the potential for zorevunersen as a disease-modifying medicine and support the proposed Phase 3 dosing regimen under discussion with global regulatory agencies."

New data will include the nine patients who received two or three initial doses of 70mg of zorevunersen in a Phase 1/2 study and then continued treatment in an open-label extension (OLE) study where they received at least two doses of 45mg. In addition, data from patients treated in the OLEs (n=73) were consistent with earlier findings and showed durable reductions in seizures and continuous improvements in multiple subdomains of the Vineland-3 through 24 months. Treatment was generally well tolerated.

Details of the Company's presentations at AES are as follows:

- **Title:** Zorevunersen (STK-001) Demonstrates Potential for Disease Modification Including Reductions in Seizures and Improvements in Cognition and Behavior in Children and Adolescents with Dravet Syndrome (DS)
  Poster Session Date & Time: Sunday, December 8 at 12:00 PM PST
  Oral Presentation Date & Time: Monday, December 9 at 3:15 PM PST
  Presenter: Linda Laux, M.D., Associate professor of Pediatrics (Neurology and Epilepsy) at Northwestern University Feinberg School of Medicine and Attending Physician at Ann & Robert H. Lurie Children's Hospital of Chicago
  Poster Number: 2.379

- **Title:** Patients with Dravet Syndrome in Open-Label Extension Studies of Zorevunersen (STK-001) Have Durable Reductions in Seizure Frequency and Clinically Meaningful Improvements in Cognition and Behavior
  Poster Session Date & Time: Sunday, December 8 at 12:00 PM PST
  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
  Poster Number: 2.364

- **Title:** Small Changes on the Vineland-3 are Meaningful to Caregivers of Patients with Dravet Syndrome
  Poster Session Date & Time: Sunday, December 8 at 12:00 PM PST
  Presenter: Carrie Condon, Director of Clinical Science at Stoke Therapeutics
  Poster Number: 3.383

- **Title:** Spectral EEG Analysis Demonstrates Decreased Slow-wave Activity in Patients with Dravet Syndrome after Treatment with Zorevunersen, an Antisense Oligonucleotide
  Poster Session Date & Time: Monday, December 9 at 12:00 PM PST
  Presenter: Nigel Colenbier, Senior Data Scientist, Epilog, Clouds of Care NV
  Poster Number: 3.407

Stoke Therapeutics Corporate Symposium

Stoke Therapeutics will host a symposium for clinicians to review new data from the Dravet syndrome program and discuss the clinical need for disease-modifying therapies that extend beyond seizure reduction. The session will focus on the behavioral, cognitive, and seizure-related impacts of the disease and what improvements are considered meaningful for clinicians and caregivers.

Title: The Potential of Disease-Modifying Treatments for Dravet Syndrome: Entering a New Era

Date and Time: Sunday, December 8, 6:00-9:00 PM PST (9:00 PM-12:00 AM EST)
Presenters: Joseph Sullivan, Andreas Brunklaus, Ingrid Scheffer, Helen Cross

Stoke Therapeutics Investor and Analyst Virtual Event

Stoke will also host a virtual event led by leading clinicians and patient advocates to discuss the effects of Dravet syndrome, the current treatment landscape, including the latest data from studies of zorevunersen, and the potential real-world impacts of a disease-modifying medicine. The presentation will be conducted virtually and include an opportunity for research analysts to ask questions of the clinician presenters.

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, 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, 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 (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 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 timing and expected progress of clinical trials, data readouts, regulatory meetings, regulatory decisions and other presentations, and the participation of scientists associated with Stoke making presentations at AES 2024 and the presentation of data at AES 2024. 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 do not fully materialize or prove incorrect, could cause our results to differ materially from those expressed or implied by such forward-looking statements. Forward-looking statements are subject to risks and uncertainties that may cause the Company's actual activities or results to differ significantly from those expressed in any forward-looking statement, including 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; 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

Source: Stoke Therapeutics, Inc.