Stoke Therapeutics to Present Data from the Company's Dravet Syndrome Program at the American Epilepsy Society 2022 Annual Meeting

November 29, 2022

– Seven abstracts related to the Company's work in Dravet syndrome will be presented, including data from a combined interim analysis of the Phase 1/2a MONARCH and ADMIRAL studies of STK-001 –

BEDFORD, Mass.--(BUSINESS WIRE)--Nov. 29, 2022-- Stoke Therapeutics, Inc. (Nasdaq: STOK), a biotechnology company dedicated to addressing the underlying cause of severe diseases by upregulating protein expression with RNA-based medicines, today announced that seven abstracts related to the Company's work in Dravet syndrome have been accepted for presentation at the American Epilepsy Society (AES) 2022 Annual Meeting, taking place December 2 – 6, in Nashville, Tennessee. The company is advancing STK-001 as potentially the first medicine to treat the underlying cause of Dravet syndrome.

"Topline results from a recent interim analysis showed marked reductions in seizure frequency, which support our work to develop STK-001 as potentially the first disease modifying medicine for Dravet syndrome," said Barry Ticho, M.D., Ph.D., Chief Medical Officer of Stoke Therapeutics. "We look forward to sharing more details from this interim analysis, along with several other presentations with researchers, clinicians and the Dravet syndrome community at the upcoming AES meeting."

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

The same data from a combined analysis of MONARCH and ADMIRAL will be presented in two posters.

- **Title:** ADMIRAL: A Phase 1/2a UK Study Investigating the Safety and Pharmacokinetics (PK) of STK-001, an Antisense Oligonucleotide (ASO), in Children and Adolescents with Dravet Syndrome (DS)
  Session Date & Time: Saturday, December 3 at 12:00 PM CST
  Presenter: Helen Cross, MB ChB, Ph.D., Professor, The Prince of Wales's Chair of Childhood Epilepsy and Head of the Developmental Neuroscience Programme at University College London Great Ormond Street Institute of Child Health, Honorary Consultant in Paediatric Neurology, President of the International League Against Epilepsy
  Poster Number: 1.215

- **Title:** MONARCH Interim Analyses: A Phase 1/2a U.S. Study Investigating Safety and Drug Exposure of STK-001, an Antisense Oligonucleotide (ASO), in Children and Adolescents with Dravet Syndrome (DS)
  Session Date & Time: Saturday, December 3 at 12:00 PM CST
  Presenter: Linda Laux, M.D., Associate Professor of Pediatrics (Neurology and Epilepsy) atNorthwestern University Feinberg School of Medicine and Attending Physician at Ann & Robert H. Lurie Children's Hospital of Chicago
  Poster Number: 1.227

Five additional posters from the Company's Dravet syndrome program will be presented.

- **Title:** SWALLOWTAIL: An Open-Label Extension (OLE) Study for Children and Adolescents with Dravet Syndrome (DS) who Previously Participated in a Study of Antisense Oligonucleotide (ASO) STK-001
  Session Date & Time: Saturday, December 3 at 12:00 PM CST
  Presenter: Colin Roberts, M.D., Director of the Doernbecher Childhood Epilepsy Program atOregon Health & Science University
  Poster Number: 1.216

- **Title:** Utilization of Pharmacokinetic (PK) Model for STK-001 in Patients with Dravet Syndrome (DS) to Predict Pharmacological Active Dose in Clinic
  Session Date & Time: Saturday, December 3 at 12:00 PM CST
  Presenter: Meena, Ph.D., Senior Vice President of Bioanalytical, DMPK and Biomarker Development at Stoke Therapeutics
  Poster Number: 1.134

- **Title:** Twelve-month Analysis of BUTTERFLY: An Observational Study to Investigate Cognition and Other Non-seizure Comorbidities in Children and Adolescents with Dravet Syndrome (DS)
  Session Date & Time: Saturday, December 3 at 12:00 PM CST
  Presenter: Joseph Sullivan, M.D., Professor of Neurology and Pediatrics and Director of the University of California San

Francisco Pediatric Epilepsy Center of Excellence
Poster Number: 1.228

- **Title:** Quantitative EEG Analysis Patients with Dravet Syndrome (DS) Treated in the Phase 1/2a MONARCH Study of STK-001, an Antisense Oligonucleotide (ASO)
  Session Date & Time: Monday, December 5 at 12:00 PM CST
  Presenter: Kimberly Parkerson, M.D., Ph.D., Vice President, Head of Neurology Clinical Development at Stoke Therapeutics
  Poster Number: 3.225

- **Title:** STK-001 Surrogate Restores the Excitability of Parvalbumin-positive Fast-spiking Interneurons in a Mouse Model of Dravet Syndrome
  Session Date & Time: Monday, December 5 at 12:00 PM CST
  Presenter: Luis Lopez-Santiago, Ph.D., Associate Research Scientist at University of Michigan Medical School
  Poster Number: 3.050

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 STK-001

STK-001 is an investigational new medicine for the treatment of Dravet syndrome currently being evaluated in ongoing clinical trials. Stoke believes that STK-001, a proprietary antisense oligonucleotide (ASO), has the potential to be the first disease-modifying therapy to address the genetic cause of Dravet syndrome. STK-001 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. Stoke has generated preclinical data demonstrating proof-of-mechanism and proof-of-concept for STK-001. STK-001 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 Phase 1/2a MONARCH Study (United States)

The MONARCH study is a Phase 1/2a open-label study of children and adolescents ages 2 to 18 who have an established diagnosis of Dravet syndrome and have evidence of a genetic mutation in the *SCN1A* gene. The primary objectives for the study are to assess the safety and tolerability of STK-001, as well as to determine the pharmacokinetics in plasma and exposure in cerebrospinal fluid. A secondary objective is to assess the efficacy as an adjunctive antiepileptic treatment with respect to the percentage change from baseline in convulsive seizure frequency. Stoke also intends to measure non-seizure aspects of the disease, such as quality of life, as secondary endpoints. Additional information about the MONARCH study can be found at https://www.monarchstudy.com/.

Patients who participated in the MONARCH study and meet study entry criteria are eligible to continue treatment in SWALLOWTAIL, an open-label extension (OLE) study designed to evaluate the long-term safety and tolerability of repeat doses of STK-001. We expect that SWALLOWTAIL will also provide valuable information on the preliminary effects of STK-001 on seizures along with non-seizure aspects of the disease, such as quality of life and cognition.

Enrollment and dosing in SWALLOWTAIL are underway.

About Phase 1/2a ADMIRAL Study (United Kingdom)

The ADMIRAL study is a Phase 1/2a open-label study of children and adolescents ages 2 to <18 who have an established diagnosis of Dravet syndrome and have evidence of a genetic mutation in the *SCN1A* gene. The primary objectives for the study are to assess the safety and tolerability of multiple doses of STK-001, as well as to determine the pharmacokinetics in plasma and exposure in cerebrospinal fluid. A secondary objective is to assess the effect of multiple doses of STK-001 as an adjunctive antiepileptic treatment with respect to the percentage change from baseline in convulsive seizure frequency. Stoke also intends to measure non-seizure aspects of the disease, such as overall clinical status and quality of life, as secondary endpoints. Additional information about the ADMIRAL study can be found at https://www.admiralstudy.com.

Patients who participated in the ADMIRAL study and meet study entry criteria are eligible to continue treatment in LONGWING, an open-label extension (OLE) study designed to evaluate the long-term safety and tolerability of repeat doses of STK-001. We expect that LONGWING will also provide valuable information on the preliminary effects of STK-001 on seizures along with non-seizure aspects of the disease, such as quality of life and cognition.

Enrollment and dosing in LONGWING are underway.

About Stoke Therapeutics

Stoke Therapeutics (Nasdaq: STOK), is a biotechnology company dedicated to addressing the underlying cause of severe diseases by upregulating protein expression with RNA-based medicines. 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, 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/ or follow Stoke on Twitter at @StokeTx.

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 STK-001 to treat the underlying causes of Dravet syndrome and reduce seizures or show improvements in non-seizure comorbidities, the participation of scientists associated with Stoke making presentations at AES 2022 and the presentation of data at AES 2022, and the ability of TANGO to design medicines to increase protein production and the expected benefits thereof. 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 its product candidates, obtain regulatory approval of and ultimately commercialize its product candidates, the timing and results of preclinical and clinical trials, the risk that positive results in a clinical trial may not be replicated in subsequent trials or success in early stage clinical trials may not be predictive of results in later stage trials and preliminary interim data readouts of ongoing trials may show results that change when such trials are completed, the Company's ability to fund development activities and achieve development goals, the Company's ability to protect intellectual property, the risks associated with the direct and indirect impacts of the ongoing COVID-19 pandemic on our business, 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, 2021, its Quarterly Reports on Form 10-Q, and the other documents the Company 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.

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