Stoke Therapeutics to Present New Preclinical Data on STK-001 at the American Epilepsy Society Annual Meeting

November 25, 2019

-- STK-001 is a potential new disease-modifying medicine for the treatment of Dravet syndrome, a severe and progressive form of genetic epilepsy --

BEDFORD, Mass.--(BUSINESS WIRE)--Nov. 25, 2019-- Stoke Therapeutics, Inc. (Nasdaq: STOK), a biotechnology company pioneering a new way to treat the underlying cause of severe genetic diseases by precisely upregulating protein expression, today announced that it will present new preclinical data on STK-001, a potential new disease-modifying medicine for the treatment of Dravet syndrome, at the American Epilepsy Society (AES) Annual Meeting, taking place December 6-10, 2019 in Baltimore.

Data will be presented from preclinical studies demonstrating the effects of STK-001, a proprietary antisense oligonucleotide (ASO), in the *Scn1a*-linked Dravet syndrome mouse model and in non-human primates. New results of EEG recordings used to measure the frequency of seizures in Dravet syndrome mice treated with STK-001 compared to placebo will be presented, as well as data on STK-001 biodistribution, target engagement, pharmacodynamics, safety and tolerability in non-human primates.

Dravet syndrome is a severe and progressive form of genetic epilepsy that affects approximately 35,000 people in the United States, Canada, Japan, Germany, France and the United Kingdom. Approximately 85% of Dravet syndrome cases are caused by spontaneous, heterozygous loss of function mutations in the *SCN1A* gene, resulting in 50% $Na_V1.1$ protein expression.

"These data support our belief that by restoring the $Na_V1.1$ protein to physiological levels, STK-001 has the potential to provide a gene-specific, disease-modifying therapy for people living with Dravet syndrome," said Edward M. Kaye, M.D., Chief Executive Officer of Stoke Therapeutics. "We look forward to continuing to advance STK-001 toward the clinic and, in the meantime, to sharing and discussing these important new data with the Dravet community at AES."

Details on the presentations are as follows:

Presentation Title: Targeted Augmentation of Nuclear Gene Output (TANGO) of *SCN1A* Prevents SUDEP in a Mouse Model of Dravet Syndrome
Session Date & Time: Saturday, December 7, 2019, 12:00 p.m. – 6:00 p.m. ET
Session Title: Poster Session 1
Presenter: Lori Isom, Ph.D., Maurice H. Seevers Professor and Chair of Pharmacology, University of Michigan Medical School
Poster Number: 1.116
Location: The Baltimore Convention Center, Hall E

Presentation Title: TANGO Oligonucleotides for the Treatment of Dravet Syndrome: Safety, Biodistribution and Pharmacology in the Non-Human Primate
Session Date & Time: Sunday, December 8, 2019, 10:00 a.m. – 4:00 p.m. ET
Session Title: Poster Session 2
Presenter: Anne Christiansen, Ph.D., Associate Director, Neuroscience, Stoke Therapeutics
Poster Number: 2.195
Location: The Baltimore Convention Center, Hall E

The abstracts for these presentations are now available online on the Events and Presentations section of Stoke's website at https://investor.stoketherapeutics.com/ or through the AES 2019 Annual Meeting mobile application.

About STK-001

STK-001 is an investigational new medicine for the treatment of Dravet syndrome. 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 $Na_V1.1$ protein expression by leveraging the non-mutant (wild-type) copy of the *SCN1A* gene to restore physiological $Na_V1.1$ levels, thereby reducing both occurrence of seizures and significant non-seizure comorbidities. Stoke has generated preclinical data demonstrating proof-of-mechanism for STK-001. STK-001 has been granted orphan drug designation by the U.S. Food and Drug Administration as a potential new treatment for Dravet syndrome.

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 cognitive regression or developmental stagnation, ataxia, speech impairment and sleep disturbances. 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 affects approximately 35,000 people in the United States, Canada, Japan, Germany, France and the United Kingdom, and it is not concentrated in a particular geographic area or ethnic group.

About Stoke Therapeutics

Stoke Therapeutics, Inc. (Nasdaq: STOK), is a biotechnology company pioneering a new way to treat the underlying causes of severe genetic diseases by precisely upregulating protein expression to restore target proteins to near normal levels. Stoke aims to develop the first precision medicine platform to target the underlying cause of a broad spectrum of genetic diseases in which the patient has one healthy copy of a gene and one mutated copy that fails to produce a protein essential to health. These diseases, in which loss of approximately 50% of normal protein expression causes disease, are called autosomal dominant haploinsufficiencies. The company's lead investigational new medicine is STK-001, a proprietary antisense oligonucleotide (ASO) that has the potential to be the first disease-modifying therapy to address the genetic cause of Dravet syndrome, a severe and progressive genetic epilepsy. Stoke is headquartered in Bedford, Massachusetts with offices in Cambridge, Massachusetts. For more information, visit https://www.stoketherapeutics.com/ or follow the company on Twitter at @StokeTx.

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: Stoke's ability to use study data to advance the development of STK-001; the ability of STK-001 to treat the underlying causes of Dravet syndrome; and the ability of TANGO to design medicines to increase protein production. Statements including words such as "plan," "continue," "expect," 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 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 Stoke'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 develop, obtain regulatory approval for and commercialize STK-001 and its future product candidates, the timing and results of preclinical studies and clinical trials, the company's ability to protect intellectual property; and other risks set forth in our filings with the Securities and Exchange Commission, including the risks set forth in our quarterly report on Form 10-Q for the quarter and nine months ended September 30, 2019. These forward-looking statements speak only as of the date hereof and Stoke specifically disclaims any obligation to update these forward-looking statements or reasons why actual results might differ, whether as a result of new information, future events or otherwise, except as required by law.

View source version on businesswire.com: https://www.businesswire.com/news/home/20191125005585/en/

Source: Stoke Therapeutics, Inc.

Stoke Media & Investor:
Dawn Kalmar
Vice President, Head of Corporate Affairs
dkalmar@stoketherapeutics.com
781-303-8302