Stoke Therapeutics Granted FDA Orphan Drug Designation for STK-001, an Investigational New Treatment for Dravet Syndrome

August 6, 2019

BEDFORD, Mass.--(BUSINESS WIRE)--Aug. 6, 2019-- Stoke Therapeutics, Inc., (Nasdaq: STOK), a biotechnology company pioneering a new way to treat the underlying cause of genetic diseases by precisely upregulating protein expression, today announced that the U.S. Food and Drug Administration (FDA) has granted orphan drug designation to its lead product candidate, STK-001, an investigational new treatment for 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. 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. Approximately 85% of Dravet syndrome cases are caused by spontaneous, heterozygous loss of function mutations in the *SCN1A* gene. This gene encodes the voltage-gated sodium channel type 1 alpha subunit ($Na_V1.1$). Currently available treatments do not address this mutation, the underlying cause of Dravet syndrome.

“The need for a medicine that will treat the underlying cause of Dravet syndrome is clear to anyone who has seen the devastating effects of the disease, and the significant impact it has on patients and their families,” said Barry S. Ticho, M.D., Chief Medical Officer of Stoke Therapeutics. “Our goal with STK-001 is to slow or even stop disease progression by treating the underlying cause of Dravet syndrome. STK-001 is designed to selectively upregulate one allele of the *SCN1A* gene to restore the protein expression to near-normal levels. We are on track to submit our investigational new drug application for STK-001 to the FDA in early 2020 and to begin a Phase 1/2 clinical study in the first half of the year.”

The FDA’s Office of Orphan Drug Products grants orphan status to support the development of medicines for safe and effective treatment, diagnosis or prevention of rare diseases or disorders that affect fewer than 200,000 people in the United States. Orphan drug designation entitles recipients to various development incentives, including tax credits for qualified clinical testing, an exemption from FDA application fees, and a seven-year period of marketing exclusivity in the United States, if the drug is approved.

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. 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 across 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 STK-001

STK-001 is an investigational new medicine for the treatment of Dravet syndrome. Stoke believes that STK-001, a proprietary antisense oligonucleotide, has the potential to be the first disease-modifying therapy to address the underlying genetic cause of Dravet syndrome. STK-001 is designed to upregulate $Na_V1.1$ protein expression from 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.

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. 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.

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 anticipated benefits associated with Orphan Drug Designation; Stoke’s expectation about timing and execution of anticipated milestones, including IND submission; the planned initiation of Stoke’s Phase 1/2 trial, and 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 Stoke 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. 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.

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Source: Stoke Therapeutics, Inc.

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