Stoke Therapeutics Enrolls First Patient in an Observational Study of Children and Adolescents Living with Dravet Syndrome

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Dravet syndrome is a severe and progressive genetic epilepsy generally caused by mutations in the SCN1A gene

The BUTTERFLY study is designed to evaluate seizure frequency and non-seizure comorbidities; data will support clinical development plans for the company's lead therapeutic candidate, STK-001

BEDFORD, Mass.--(BUSINESS WIRE)--Aug. 20, 2019-- Stoke Therapeutics, Inc. (Nasdaq: STOK), a biotechnology company that is pioneering a new way to treat the underlying cause of genetic diseases by precisely upregulating protein expression, today announced enrollment of the first patient in an observational study of children and adolescents ages 2 to 18 with 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.

BUTTERFLY is a two-year observational study that is designed to evaluate seizure frequency and non-seizure comorbidities associated with the disease, including motor and speech impairment, intellectual and developmental disabilities, behavioral deficits and abnormal sleep patterns. Data from the study will support clinical development plans for the company's lead therapeutic candidate, STK-001, an investigational new treatment for Dravet syndrome.

"Our goal is to develop the first medicine to treat the underlying cause of Dravet syndrome," said Barry Ticho, M.D., Ph.D., Chief Medical Officer of Stoke Therapeutics. "Although this study will not evaluate potential new medicines for Dravet, we believe it will provide important information about the range of effects of the disease on children and young adults that will be useful in planning for the clinical development of STK-001, our lead therapeutic candidate."

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 the underlying cause of Dravet syndrome. Even when taking the current standard-of-care regimen, which includes multiple antiepileptic medicines, more than 90% of patients still report suffering from incomplete seizure control.

"Increased awareness along with more widespread availability of genetic testing have allowed us to diagnose genetic epilepsies like Dravet syndrome earlier, allowing physicians to help patients and their families better manage the disease," said Joseph Sullivan, M.D., professor of Neurology at the University of California San Francisco and director of the Benioff Children's Hospital Pediatric Epilepsy Center of Excellence. "We have made significant progress in understanding the genetic basis of many childhood epilepsies, but we still do not completely understand the full spectrum or impact of the disease. This study will give us new insights that will help us better care for our patients and improve our research and development efforts for potential new medicines with the goal of improving outcomes beyond seizure control."

About the BUTTERFLY Study

The BUTTERFLY study is an observational study of children and adolescents ages 2 to 18 who have been diagnosed with Dravet syndrome as a result of an *SCN1A* gene mutation. The study is designed to evaluate seizure frequency and non-seizure comorbidities associated with the disease, including motor and communication impairment, intellectual and developmental disabilities, behavioral deficits and abnormal sleep patterns. No investigational medications or other treatments will be provided. Participants will continue to receive their usual care, and they will be observed by a team of doctors and nurses over time for up to two years. The study is expected to enroll at approximately 20 sites in the United States. For more information about enrollment, please email BUTTERFLYStudy@iqvia.com.

Additional Information 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 (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 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. 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 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: enrollment in the BUTTERFLY study and the study's ability to support Stoke's clinical development plans; Stoke's expectation about timing and execution of anticipated milestones; 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 and uncertainties described under the heading "Risk Factors" in 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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