Stoke Therapeutics Reports First Quarter Financial Results and Provides Business Updates

May 15, 2020

– Company on track to begin enrollment and dosing of STK-001 in Part A of Phase 1/2a "Monarch" clinical trial in children and adolescents with Dravet syndrome in 2H 2020 –

– Research activities ongoing to identify an additional preclinical candidate derived from the company's TANGO technology platform for the treatment of an additional genetic disease in 2H 2020–

– As of March 31, 2020, company has $211.5 million in cash, cash equivalents and restricted cash, anticipated to fund operations into 2023 –

BEDFORD, Mass.--(BUSINESS WIRE)--May 15, 2020-- 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 reported financial results for the first quarter of 2020 and provided business updates.

"I am incredibly gratified by the focus and determination of our employees during these challenging times. Thanks to their unwavering commitment to patients, we are continuing to make progress with STK-001 and are on track to enroll and dose the first children and adolescents with Dravet syndrome in the Phase 1/2a Monarch study later this year," said Edward M. Kaye, M.D., Chief Executive Officer of Stoke Therapeutics. "Our understanding of the potential for our TANGO technology in additional genetic diseases has continued to advance and we are generating data that we believe will support the nomination of a second preclinical candidate in the second half of 2020."

First Quarter 2020 Business Highlights and Recent Developments

- As previously announced, Stoke received U.S. Food and Drug Administration (FDA) clearance to begin dosing in Part A of its planned Phase 1/2a "Monarch" study of STK-001 in children and adolescents ages 2 to 18 years old with Dravet syndrome. Part A of the study is designed to evaluate two dose cohorts of STK-001. There is a partial clinical hold on Part B of the study, which is designed to evaluate higher doses. Stoke has initiated preclinical studies to address the FDA's request to more fully characterize the safety profile of STK-001 at doses higher than the current no observed adverse effect level. This partial clinical hold is not due to any identified manufacturing or safety issue.
- On May 12, 2020 Stoke presented the first in-vivo proof-of-concept data for TANGO antisense oligonucleotides (ASO) in an ocular disease at the American Society of Gene & Cell Therapy (ASGCT) 23rd Annual Meeting. The preclinical data showed in-vitro and in-vivo target engagement and protein upregulation in *OPA1* protein deficiency, which is the primary cause of autosomal dominant optic atrophy (ADOA), the most common inherited optic nerve disorder.
- Patients currently enrolled in the BUTTERFLY observational study are continuing to be followed. We have experienced a slowing of new patient enrollment in this study due to the impact of COVID-19 on clinical trial sites.

Upcoming Anticipated Milestones

- Data on the use of Stoke's TANGO technology to address *OPA1* protein deficiency are planned for virtual presentation at the Association for Research in Vision and Ophthalmology (ARVO) Annual Meeting in June.
- Enrollment and dosing of patients in Part A of the Phase 1/2a Monarch single-ascending dose study of STK-001 in children and adolescents with Dravet syndrome is still expected to begin in the second half of 2020. Currently, we have not experienced any delay in initiating Monarch. To help mitigate the impact of COVID-19 to our clinical trial, we are pursuing innovative approaches such as remote monitoring and remote patient visits. We continue to anticipate preliminary data from the study in 2021.
- Nomination of a second product candidate for the treatment of an additional genetic disease is expected in the second half of 2020.

First Quarter and Year-to-Date Results

- Net loss for the first three months of 2020 was $11.0 million, compared to net loss of $5.7 million for the same period in 2019.
- Research and development expenses for the three months ended March 31, 2020 were $7.2 million, compared to $4.1 million for the same period in 2019.
- General and administrative expenses for the three months ended March 31, 2020 were $4.5 million, compared to $2.2 million for the same period in 2019.
- The increase in expenses for the 2020 period over the same period in 2019 primarily relate to increases in costs associated with personnel, third party contracts, consulting, facilities and others associated with development activities for

STK-001, research on additional therapeutics and growing a public corporation.

- As of March 31, 2020, Stoke had approximately $211.5 million in cash, cash equivalents and restricted cash, which is anticipated to fund operations into 2023.

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 and proof-of-concept for STK-001. STK-001 has been granted orphan drug designation by the FDA as a potential new treatment for Dravet syndrome.

About Phase 1/2a Clinical Study (Monarch)

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 pathogenic genetic mutation in the SCN1A gene. The primary objectives for the study will be to assess the safety and tolerability of STK-001, as well as to characterize human pharmacokinetics. A secondary objective will be to assess the efficacy as an adjunctive antiepileptic treatment with respect to the percentage change from baseline in convulsive seizure frequency over a 12-week treatment period. Stoke also intends to measure non-seizure aspects of the disease, such as quality of life as secondary endpoints. Stoke plans to enroll approximately 40 patients at 20 sites in the United States. Enrollment and dosing are expected to begin in the second half of 2020.

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 severe intellectual disabilities, severe developmental disabilities, motor impairment, speech impairment, autism, behavioral difficulties and sleep abnormalities. 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 (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: our first quarter results; the direct and indirect impact of COVID-19 on our business, financial condition and operations, including on our, expenses, supply chain, strategic partners, research and development costs, clinical trials and employees; our expectation about timing and execution of anticipated milestones, including enrollment in Part A of our Phase 1/2a Monarch clinical trial in Dravet syndrome, and our 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. These forward-looking statements may be accompanied by such words as “aim,” “anticipate,” “believe,” “could,” “estimate,” “expect,” “forecast,” “goal,” “intend,” “may,” “might,” “plan,” “potential,” “possible,” “will,” “would,” and other words and terms of similar meaning. 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. These statements involve risks and uncertainties that could cause actual results to differ materially from those reflected in such statements, including: our ability to develop, obtain regulatory approval for and commercialize STK-001 and future product candidates; the timing and results of preclinical studies 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 clinical trials; risks associated with clinical trials, including our ability to adequately manage clinical activities, unexpected concerns that may arise from additional data or analysis obtained during clinical trials, regulatory authorities may require additional information or further studies, or may fail to approve or may delay approval of our drug candidates; the occurrence of adverse safety events; failure to protect and enforce our intellectual property, and other proprietary rights; failure to successfully execute or realize the anticipated benefits of our strategic and growth initiatives; risks relating to technology failures or breaches; our dependence on collaborators and other third parties for the development, regulatory approval, and commercialization of products and other aspects of our business, which are outside of our full control; risks associated with current and potential delays, work stoppages, or supply chain disruptions caused by the coronavirus pandemic; risks associated with current and potential future healthcare reforms; risks relating to attracting and retaining key personnel; failure to comply with legal and regulatory requirements; risks relating to access to capital and credit markets; environmental risks; risks relating to the use of social media for our business; and the other risks and uncertainties that are described in the Risk Factors section of our most recent annual or quarterly report and in other reports we have filed with the U.S. Securities and Exchange Commission. These statements are based on our current beliefs and expectations and speak only as of the date of this press release. We do not undertake any obligation to publicly update any forward-looking statements.

Financial Tables Follow

Stoke Therapeutics, Inc.
Condensed consolidated balance sheets
(in thousands, except share and per share amounts)

(unaudited)

	March 31, 2020	December 31, 2019
Assets		
Current assets:		
Cash and cash equivalents	$211,288	$ 222,471
Prepaid expenses and other current assets	4,342	3,281
Interest receivable	144	281
Total current assets	$215,774	$ 226,033
Restricted cash	205	205
Operating lease right-of-use assets	1,900	—
Property and equipment, net	2,962	2,512
Total assets	$220,841	$ 228,750
Liabilities and stockholders' equity		
Current liabilities:		
Accounts payable	$ 1,779	$ 751
Accrued and other current liabilities	3,676	3,350
Total current liabilities	$ 5,455	$ 4,101
Long term liabilities	1,044	221
Total liabilities	$ 6,499	$ 4,322
Commitments and contingencies		
Stockholders' equity		
Common stock, par value of $0.0001 per share; 300,000,000 shares authorized, 32,967,350 and 32,861,842 shares issued and outstanding as of March 31, 2020 and December 31, 2019, respectively	3	3
Additional paid-in capital	283,413	282,460
Accumulated deficit	(69,074)	(58,035)
Total stockholders' equity	$214,342	$ 224,428
Total liabilities and stockholders' equity	$220,841	$ 228,750

Stoke Therapeutics, Inc.
Condensed consolidated statements of operations and comprehensive loss
(in thousands, except share and per share amounts)
(unaudited)

	Three Months Ended March 31,	
	2020	2019
Revenue	$ —	$ —
Operating expenses:		
Research and development	7,215	4,133
General and administrative	4,520	2,189
Total operating expenses	11,735	6,322
Loss from operations	(11,735)	(6,322)
Other income:		
Interest income	674	580
Other income, net	22	—
Total other income	696	580
Net loss and comprehensive loss	$ (11,039)	$ (5,742)
Net loss per share attributable to common stockholders, basic and diluted	$ (0.34)	$ (6.89)
Weighted-average common shares outstanding, basic and diluted	32,897,395	833,469

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