Stoke Therapeutics Reports Second Quarter Financial Results and Provides Business Updates

August 10, 2020

– First patient dosed with STK-001 in Part A of Phase 1/2a MONARCH clinical trial for Dravet syndrome –

– Company on track to identify an additional pre-clinical candidate derived from its TANGO platform for the treatment of an additional genetic disease in 2H 2020 –

– As of June 30, 2020, company has $202.1 million in cash, cash equivalents and restricted cash, anticipated to fund operations into 2023 –

BEDFORD, Mass.--(BUSINESS WIRE)-- Aug. 10, 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 second quarter of 2020 and provided business updates.

"Today we are announcing that the first patient has been dosed with STK-001, which we believe has the potential to be the first-disease modifying medicine for Dravet syndrome, a severe and progressive genetic epilepsy that is characterized by developmental delays and cognitive impairment, in addition to seizure activity," said Edward M. Kaye, M.D., Chief Executive Officer of Stoke Therapeutics. "The start of MONARCH also marks Stoke's official transition to a clinical-stage biotech company. We enter this new stage in a strong financial position to execute on our plans for STK-001 in Dravet syndrome and continue to advance the potential of our TANGO platform for additional genetic diseases."

Second Quarter 2020 Business Highlights and Recent Developments

- Stoke announced today that the first patient was enrolled and has been dosed with STK-001 in Part A of the Phase 1/2a MONARCH study of 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. The U.S. FDA placed a partial clinical hold on Part B of the study, which is designed to evaluate higher doses of STK-001. Stoke has generated additional data and is in the process of preparing its response to the FDA.
- On June 12, Stoke presented additional data on the use of Stoke's TANGO technology to address OPA1 protein deficiency at the Association for Research in Vision and Ophthalmology (ARVO). *OPA1* protein deficiency is the primary cause of autosomal dominant optic atrophy (ADOA), the most common inherited optic nerve disorder.
- On July 9, the journal *Nature Communications* published data supporting Stoke's Targeted Augmentation of Nuclear Gene Output (TANGO) approach to addressing severe genetic diseases by precisely upregulating protein expression.
- The BUTTERFLY observational study is ongoing. Despite experiencing a slowing in new patient enrollment earlier this year due to the impact of COVID-19, new patient enrollment has resumed and we believe we have achieved sufficient participation in the study to provide informative data about the natural progression of Dravet syndrome.

Upcoming Anticipated Milestones

- Nomination of a second product candidate for the treatment of an additional genetic disease is expected in the second half of 2020.

Second Quarter and Year-to-Date Results

- Net loss for the three months ended June 30, 2020 were $13.0 million, or $0.39 per share compared to $7.8 million or $1.54 per share for the same period in 2019.
- Research and development expenses for the three months ended June 30, 2020 were $8.0 million, compared to $6.0 million for the same period in 2019.
- General and administrative expenses for the three months ended June 30, 2020 were $5.0 million, compared to $2.4 million for the same period in 2019.
- Net loss for the first six months of 2020 was $24.0 million or $0.73 per share, compared to net loss of $13.6 million or $4.57 per share for the same period in 2019.
- Research and development expenses for the six months ended June 30, 2020 were $15.2 million, compared to $10.2 million for the same period in 2019.
- General and administrative expenses for the six months ended June 30, 2020 were $9.6 million, compared to $4.6 million for the same period in 2019.
- The increase in expenses for the three and six month periods in 2020 over the same periods 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 June 30, 2020, Stoke had approximately $202.1 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 across 20 sites in the United States.

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: future operating results, financial position and liquidity, 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, responses to regulatory authorities, expected nomination of a second product candidate and timing thereof, 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 and the expected benefits thereof. 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)

	June 30, 2020	December 31, 2019
Assets		
Current assets:		
Cash and cash equivalents	$ 201,930	$ 222,471
Prepaid expenses and other current assets	3,528	3,281
Deferred financing costs	77	—
Interest receivable	9	281
Total current assets	$ 205,544	$ 226,033
Restricted cash	205	205
Operating lease right-of-use assets	1,642	—
Property and equipment, net	2,823	2,512
Total assets	$ 210,214	$ 228,750
Liabilities and stockholders' equity		
Current liabilities:		
Accounts payable	$ 904	$ 751
Accrued and other current liabilities	4,901	3,350
Total current liabilities	$ 5,805	$ 4,101
Long term liabilities	1,009	221
Total liabilities	$ 6,814	$ 4,322
Commitments and contingencies (Note 5)		
Stockholders' equity		
Common stock, par value of $0.0001 per share; 300,000,000 shares authorized, 33,212,544 and 32,861,842 shares issued and outstanding as of June 30, 2020 and December 31, 2019, respectively	3	3
Additional paid-in capital	285,430	282,460
Accumulated deficit	(82,033)	(58,035)
Total stockholders' equity	$ 203,400	$ 224,428
Total liabilities and stockholders' equity	$ 210,214	$ 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 June 30,		Six Months Ended June 30,	
	2020	2019	2020	2019
Revenue	$ —	$ —	$ —	$ —
Operating expenses:				
Research and development	7,968	6,023	15,183	10,156
General and administrative	5,044	2,422	9,563	4,611
Total operating expenses	13,012	8,445	24,746	14,767
Loss from operations	(13,012)	(8,445)	(24,746)	(14,767)
Other income:				
Interest income	50	629	723	1,210
Other income (expense), net	3	(3)	25	(4)
Total other income	53	626	748	1,206
Net loss and comprehensive loss	$ (12,959)	$ (7,819)	$ (23,998)	$ (13,561)
Net loss per share attributable to common stockholders, basic and diluted	$ (0.39)	$ (1.54)	$ (0.73)	$ (4.57)
Weighted-average common shares outstanding, basic and diluted	33,054,656	5,083,620	32,976,026	2,970,292

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