Stoke Therapeutics Reports Full Year 2019 Financial Results and Provides Business Updates

March 23, 2020

– Company receives FDA clearance to begin dosing STK-001 in Part A of Phase 1/2a "Monarch" clinical trial in Dravet syndrome; enrollment and dosing expected to start in 2H 2020 –

– Part B of study, designed to evaluate higher doses of STK-001, on partial clinical hold pending preclinical data to more fully characterize the safety profile of STK-001 at doses higher than the current NOAEL; partial clinical hold not due to any identified manufacturing or safety issue –

– Additional toxicology studies now underway; preliminary data from Monarch study still anticipated in 2021 –

– As of December 31, 2019, company has approximately $222.7 million in cash, cash equivalents and restricted cash, which is anticipated to fund operations into 2023 –

BEDFORD, Mass.--(BUSINESS WIRE)--Mar. 23, 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 full year ended December 31, 2019, and provided business updates.

"In 2019 we advanced our understanding of the potential applications of our TANGO technology to a variety of genetic targets, and generated preclinical data that underscore our confidence in STK-001 as a promising potential disease-modifying treatment for Dravet syndrome," said Edward M. Kaye, M.D., Chief Executive Officer of Stoke Therapeutics. "As we look ahead to 2020, we are poised to become a clinical-stage company with the anticipated start of our first in-human study of STK-001, and a growing portfolio as we work toward identifying our next preclinical product candidate later this year."

Today, the company also provided an update on its lead product candidate, STK-001, for Dravet syndrome. In the first quarter of 2020, Stoke received communication from the U.S. Food and Drug Administration (FDA) confirming that it may proceed with clinical 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.

As part of that communication, the FDA also informed the company that it has placed a partial clinical hold on Part B of the study, which is designed to evaluate higher doses of STK-001, pending additional preclinical toxicology data. This partial clinical hold is not due to any identified manufacturing or safety issue. Rather, the FDA is requesting additional preclinical testing to determine the safety profile of doses higher than the current no observed adverse effect level (NOAEL).

The NOAEL was determined using data from a pivotal non-human primate study that evaluated intrathecal delivery of single dose levels of STK-001. The highest dose administered in this study was equivalent to a human dose that is higher than what Stoke plans to administer in Part B of its Phase 1/2a clinical study and did not demonstrate effects that were considered adverse. Stoke has initiated single-dose toxicology studies to more fully characterize the safety profile of STK-001 at higher doses, in order to facilitate the removal of the partial clinical hold and proceed to Part B of the study. At the completion of dosing in Part A, and upon FDA clearance, Stoke will proceed with the higher-dosing cohorts planned in Part B of the study. Stoke still anticipates preliminary data from the study in 2021 and is working to minimize any potential delay to continued clinical testing of STK-001.

"We are encouraged by the preclinical STK-001 safety and efficacy data generated to date," said Barry Ticho, M.D., Ph.D., Chief Medical Officer of Stoke Therapeutics. "We look forward to moving ahead with our plans to enroll and dose patients with Dravet syndrome in Part A of the Phase 1/2a study later this year. To facilitate removal of the partial clinical hold on Part B of the study, we have begun additional single-dose toxicology studies and will work closely with the FDA to address their request and move forward as soon as possible with Part B."

Fourth Quarter 2019 Business Highlights:

- Presented new preclinical data supporting the planned clinical development of STK-001 at the American Epilepsy Society meeting in Baltimore, December 6 to 8, 2019. Highlights from these data included significant improvements in survival and reductions in seizure frequency as measured by electroencephalogram (EEG) in a mouse model of Dravet syndrome. Data from studies in non-human primates showed distribution throughout the brain, target engagement and increased $Na_v1.1$ protein expression throughout the cortex after a single intrathecal injection. Safety findings showed STK-001 to be well-tolerated at both dose levels studied.
- Added to the Nasdaq Biotechnology Index on December 23, 2019.
- Submitted an Investigational New Drug (IND) application for STK-001 to the FDA in late 2019.

Upcoming Anticipated Milestones:

- An abstract detailing the use of TANGO antisense oligonucleotides (ASO) to address the underlying OPA1 protein deficiency that causes autosomal dominant optic atrophy, a rare syndrome that causes vision loss, was previously accepted for presentation at the Association for Research in Vision and Ophthalmology (ARVO) Annual Meeting in May

2020. Following cancellation of the meeting due to COVID-19, Stoke is planning to submit the abstract for review and presentation at a later meeting.
- 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 in the second half of 2020.
- Nomination of a second product candidate for the treatment of an additional genetic disease expected in the second half of 2020.

Year End 2019 Results

- Net loss for the year ended December 31, 2019 was $32.3 million, compared to net loss of $12.5 million for the same period in 2018.
- Research and development expenses for the year ended December 31, 2019 were $23.8 million, compared to $8.4 million for the same period in 2018.
- General and administrative expenses for the year ended December 31, 2019 were $11.9 million, compared to $4.4 million for the same period in 2018.
- The increases in expenses for the 2019 periods over the same periods in 2018 primarily relate to increases in costs associated with personnel costs, third-party contracts, consulting, facilities and other costs associated with development activities for STK-001, research on additional therapeutics and growing a public corporation.
- As of December 31, 2019, Stoke had approximately $222.7 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 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 year end results; our expectation about timing and execution of anticipated milestones, including our IND submission; the planned initiation of 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)

	As of December 31,	
	2019	**2018**
Assets		
Current assets:		
Cash and cash equivalents	$ 222,471	$ 105,399
Prepaid expenses and other current assets	3,281	548
Interest receivable	281	196
Total current assets	226,033	106,143
Restricted cash	205	204
Property and equipment, net	2,512	1,192
Total assets	$ 228,750	$ 107,539
Liabilities and stockholders' equity		
Current liabilities:		
Accounts payable	$ 751	$ 1,071
Accrued and other current liabilities	3,350	1,396
Total current liabilities	4,101	2,467
Long term liabilities	221	4
Total liabilities	4,322	2,471
Commitments and contingencies		
Stockholders' equity		
Preferred Stock, par value of $0.0001 per share; 10,000,000 shares authorized, none issued and outstanding as of December 31, 2019; and no shares authorized, issued or outstanding as of December 31, 2018	—	—
Convertible Preferred Stock, par value of $0.0001 per share; no shares authorized, issued or outstanding at December 31, 2019; 225,584,874 shares authorized, 22,677,585 shares issued and outstanding as of December 31, 2018, aggregate liquidation preference of $130,850 at December 31, 2018	—	2
Common stock, par value of $0.0001 per share; 300,000,000 and 278,527,249 shares authorized, 32,861,842 and 727,413 shares issued and outstanding as of December 31, 2019 and 2018, respectively	3	—
Additional paid-in capital	282,460	130,776
Accumulated deficit	(58,035)	(25,710)
Total stockholders' equity	224,428	105,068
Total liabilities and stockholders' equity	$ 228,750	$ 107,539

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

	Year Ended December 31,	
	2019	**2018**
Revenue	$ —	$ —
Operating expenses:		
Research and development	23,764	8,371
General and administrative	11,914	4,410

Total operating expenses	35,678	12,781
Loss from operations	(35,678)	(12,781)
Other income (expense):		
Interest income	3,351	270
Other income, net	2	(10)
Total other income (expense)	3,353	260
Net loss and comprehensive loss	(32,325)	(12,521)
Net loss per share attributable to common stockholders—basic and diluted	$ (1.80)	$ (17.65)
Weighted average common shares outstanding—basic and diluted	17,971,443	709,336

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