Stoke Therapeutics Reports Full Year 2020 Financial Results and Provides Business Updates

March 9, 2021

– First patient dosed in the multiple ascending dose portion of Phase 1/2a MONARCH study of STK-001 in Dravet syndrome –

– Preliminary safety and PK data from the single ascending dose portion of MONARCH still expected in H2 2021 –

– As of December 31, 2020, Company had $287.5 million in cash, cash equivalents and restricted cash, anticipated to fund operations into 2024 –

BEDFORD, Mass.--(BUSINESS WIRE)--Mar. 9, 2021-- Stoke Therapeutics, Inc. (Nasdaq: STOK), a biotechnology company dedicated to addressing the underlying cause of severe diseases by up-regulating protein expression with RNA-based medicines, today reported financial results for the full year ended December 31, 2020 and provided business updates.

"During the last year, the power and potential of RNA medicines became clear to the world. Simultaneously, the Stoke team advanced our efforts to discover and develop RNA-based medicines that aim to address the underlying cause of severe genetic diseases by selectively boosting protein production," said Edward M. Kaye, M.D., Chief Executive Officer of Stoke Therapeutics. "Our Phase 1/2a MONARCH study in children and adolescents with Dravet syndrome is well underway and patients are now being treated with STK-001 in both the single and multiple ascending dose portions of this study. We also recently initiated an open-label extension study called SWALLOWTAIL that will provide ongoing treatment with STK-001 for patients who complete MONARCH. We expect that SWALLOWTAIL and the MAD portion of MONARCH will provide important information on the potential effects of repeat doses of STK-001."

Dr. Kaye continued, "Looking ahead to 2021, we are on track to report preliminary safety and PK data from the SAD portion of MONARCH in the second half of the year. Our pipeline continues to advance as well. Lead optimization efforts are underway to identify a clinical candidate for OPA1 protein deficiency, the primary cause of autosomal dominant optic atrophy, and our research team is interrogating several targets of interest with the goal of demonstrating *in vivo* proof of mechanism and safety for another program by year end."

Fourth Quarter 2020 Business Highlights and Recent Developments

- The Company announced today continued clinical progress with the Phase 1/2a MONARCH study of children and adolescents with Dravet syndrome. Enrollment of patients in the first two single ascending dose (SAD) cohorts (10mg and 20mg) is now complete. Enrollment and dosing in the third (30mg) SAD cohort is underway.
- Also announced today was the start of the multiple ascending dose (MAD) portion of the MONARCH study with the first patient enrolled and dosed in February at the 20mg dose level.
- In January 2021, the Company began dosing patients in SWALLOWTAIL, an open label extension (OLE) study designed to evaluate the long-term safety and tolerability of repeat doses of STK-001 in patients who participated in the Phase 1/2a MONARCH study.
- In January 2021, the Company announced completion of enrollment (n=36) in the BUTTERFLY observational study of children and adolescents ages 2 to 18 years old with Dravet syndrome. The study is ongoing.
- In December 2020, the Company presented four abstracts related to its work in Dravet syndrome at the American Epilepsy Society (AES) 2020 Virtual Annual Meeting. Highlights from these presentations include baseline data from children and adolescents with Dravet syndrome enrolled in the BUTTERFLY observational study, which provide initial support of use of standard measures of cognition (BSID-III and WPPSI-IV) in evaluating non-seizure comorbidities in people with Dravet syndrome and new preclinical data demonstrating the ability of TANGO ASOs to help restore normal function of nerve cells, which correlate to reductions in seizure frequency and extended survival. In addition, data showed that a no-cost epilepsy genetic testing program co-sponsored by Stoke can diagnose genetic epilepsies earlier.
- On November 24, 2020, the Company closed an underwritten public offering of 2,875,000 shares of its common stock at a price to the public of $39.00 per share. The net proceeds from the offering were approximately $104.9 million, after deducting underwriting discounts, commissions and offering expenses.

Upcoming Anticipated Milestones

- Preliminary safety and pharmacokinetic data from the SAD portion of the Phase 1/2a MONARCH study are expected in the second half of 2021.
- In the second half of 2021, the Company plans to initiate natural history data collection of people living with autosomal dominant optic atrophy (ADOA) to better understand the natural progression of this disease, which is the most common inherited optic nerve disorder. The Company hopes to use the data to support future clinical development plans for the Company's *OPA1* program.
- Lead optimization is underway to potentially identify a clinical candidate for OPA1, the Company's next preclinical target, by

the end of 2021.
- Also, by the end of 2021, the Company expects to demonstrate *in vivo* proof of mechanism and safety for a third TANGO ASO program.

Year End 2020 Financial Results

- Net loss for the year ended December 31, 2020 was $52.3 million, or $1.56 per share compared to $32.3 million or $1.80 per share for the same period in 2019.
- Research and development expenses for the year ended December 31, 2020 were $32.2 million, compared to $23.8 million for the same period in 2019.
- General and administrative expenses for the year ended December 31, 2020 were $20.8 million, compared to $11.9 million for the same period in 2019.
- The increase in expenses for the 2020 periods 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 December 31, 2020, Stoke had approximately $287.5 million in cash, cash equivalents and restricted cash, which is anticipated to fund operations into 2024.

About TANGO

TANGO (Targeted Augmentation of Nuclear Gene Output) is Stoke's proprietary research platform. Stoke's initial application for this technology are diseases in which one copy of a gene functions normally and the other is mutated, also called haploinsufficiencies. In these cases, the mutated gene does not produce its share of protein, so the body does not function normally. Using the TANGO approach and a deep understanding of RNA science, Stoke researchers design antisense oligonucleotides (ASOs) that bind to pre-mRNA and help the target genes produce more protein. TANGO aims to restore missing proteins by increasing – or stoking – protein output from healthy genes, thus compensating for the non-functioning copy of the gene.

About STK-001

STK-001 is an investigational new medicine for the treatment of Dravet syndrome currently being evaluated in a Phase 1/2a clinical trial. 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 are to assess the safety and tolerability of STK-001, as well as to characterize human pharmacokinetics. A secondary objective is 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. Enrollment and dosing are ongoing in MONARCH and Stoke plans to enroll approximately 48 patients in the study across 20 sites in the United States.

Additional information about the MONARCH study can be found at https://www.monarchstudy.com/.

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. The disease is classified as a developmental and epileptic encephalopathy due to the developmental delays and cognitive impairment associated with the disease. 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 Autosomal Dominant Optic Atrophy (ADOA)

Autosomal dominant optic atrophy (ADOA) is the most common inherited optic nerve disorder. It is a rare disease that causes progressive and irreversible vision loss in both eyes starting in the first decade of life. Symptoms typically begin between the ages of 4 and 6 years old, affecting males and females equally. The severity of the condition by adolescence reflects the overall level of visual function to be expected throughout most of the individual's adult life. Roughly half of people with ADOA fail driving standards and up to 46% are registered as legally blind. ADOA is considered a haploinsufficiency, as most people living with ADOA have genetic mutations in the *OPA1* gene that result in only half the necessary *OPA1* protein being produced. More than 400 *OPA1* mutations have been reported in people diagnosed with ADOA. Currently there is no approved treatment for people living with ADOA.

About Stoke Therapeutics

Stoke Therapeutics (Nasdaq: STOK), is a biotechnology company dedicated to addressing the underlying cause of severe diseases by up-regulating protein expression with RNA-based medicines. Using the company's proprietary TANGO (Targeted Augmentation of Nuclear Gene Output) approach Stoke is developing antisense oligonucleotides (ASOs) to selectively restore protein levels. The company's first compound, STK-001 is in clinical

testing for the treatment of Dravet syndrome, a severe and progressive genetic epilepsy. Dravet syndrome is one of many diseases caused by a haploinsufficiency, in which a loss of ~50% of normal protein levels leads to disease. The company is pursuing treatment for a second haploinsufficient disease, autosomal dominant optic atrophy (ADOA), the most common inherited optic nerve disorder. Stoke's initial focus is haploinsufficiencies and diseases of the central nervous system and the eye, although proof of concept has been demonstrated in other organs, tissues, and systems, supporting the company's belief in the broad potential for its proprietary approach. 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 and cash runway; our expectation about timing and execution of anticipated milestones, responses to regulatory authorities, expected nomination of future product candidates and programs and timing thereof; preclinical data and study results regarding OPA1 and STK-001, 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, any potential clinical candidate for OPA1 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 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; 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,	
	2020	**2019**
Assets		
Current assets:		
Cash and cash equivalents	$ 287,308	$ 222,471
Prepaid expenses and other current assets	6,435	3,281
Deferred financing costs	181	—
Interest receivable	6	281
Total current assets	293,930	226,033
Restricted cash	205	205
Operating lease right-of-use assets	1,115	—
Property and equipment, net	2,675	2,512
Total assets	$ 297,925	$ 228,750
Liabilities and stockholders' equity		
Current liabilities:		
Accounts payable	$ 1,495	$ 751
Accrued and other current liabilities	9,930	3,350
Total current liabilities	11,425	4,101
Long term liabilities	422	221
Total liabilities	11,847	4,322
Commitments and contingencies		
Stockholders' equity		
Common stock, par value of $0.0001 per share; 300,000,000 shares authorized, 36,577,149 and 32,861,842 shares issued and outstanding as of December 31, 2020 and 2019, respectively	4	3
Additional paid-in capital	396,352	282,460

Accumulated deficit	(110,278)	(58,035)
Total stockholders' equity	286,078	224,428
Total liabilities and stockholders' equity	$ 297,925	$ 228,750

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

	Year Ended December 31,	
	2020	**2019**
Revenue	$ —	$ —
Operating expenses:		
Research and development	32,197	23,764
General and administrative	20,847	11,914
Total operating expenses	53,044	35,678
Loss from operations	(53,044)	(35,678)
Other income (expense):		
Interest income	744	3,351
Other income, net	57	2
Total other income (expense)	801	3,353
Net loss and comprehensive loss	(52,243)	(32,325)
Net loss per share —basic and diluted	$ (1.56)	$ (1.80)
Weighted average common shares outstanding—basic and diluted	33,488,456	17,971,443

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