Stoke Therapeutics Reports Third Quarter 2019 Financial Results and Provides Business Update

November 12, 2019

BEDFORD, Mass.--(BUSINESS WIRE)--Nov. 12, 2019-- 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 third quarter of 2019.

“Our successful IPO has put us in a strong financial position to rapidly advance STK-001 toward the clinic, invest in research to help us identify the most promising new genetic targets for our TANGO platform and continue to hire the talented team we need to get the job done,” said Edward M. Kaye, M.D., Chief Executive Officer of Stoke Therapeutics. “In August we initiated BUTTERFLY, our observational study of children and adolescents living with Dravet syndrome, which is designed to provide information about the natural course of the disease – including a range of effects beyond just seizures – that impact patient health and quality of life. We remain on track with our clinical plans for STK-001, our investigational new medicine for the treatment of Dravet syndrome. We have completed the toxicology studies for our investigational new drug application and have made good progress on the protocol for our planned Phase 1/2 study.”

Third Quarter 2019 Business Highlights:

- On August 6, 2019, we announced that the U.S Food and Drug Administration (FDA) granted orphan drug designation to our lead product candidate, STK-001, an investigational new medicine for Dravet syndrome, a severe and progressive form of epilepsy.
- On August 20, 2019, we announced first patient enrollment in BUTTERFLY, an observational study of children and adolescents ages 2 to 18 with Dravet syndrome. The two-year study is designed to evaluate non-seizure comorbidities associated with the disease, including cognitive development, behavior, movement, communication skills, seizure frequency and sleep pattern. Data from the study will help inform clinical development plans for STK-001.
- On September 11, 2019, Stoke announced the company has been added to the Russell 2000® and 3000® indexes effective September 20, 2019. Stoke’s addition is based on FTSE Russell’s quarterly update.

Recent Developments:

- In October 2019, the company expanded to more than 50 employees and announced the appointments of two new members of the senior leadership team: Robin A. Walker, J.D., as Senior Vice President and Chief Legal Officer, and Joan Wood as Head of Human Resources.

Upcoming Anticipated Milestones:

- New preclinical data on STK-001 to be presented at the American Epilepsy Society meeting in Baltimore, December 6-8, 2019.
- Submission of the investigational new drug (IND) application for STK-001 to the FDA in early 2020.
- Initiation of Phase 1/2 single-ascending dose study of STK-001 in children and adolescents with Dravet syndrome expected in the first half of 2020.
- Nomination of second product candidate for the treatment of an additional genetic disease expected by the first half of 2020.

Third Quarter and Year to Date 2019 Results

- Net loss for the third quarter of 2019 was $8.6 million, compared to net loss of $3.3 million for the same period in 2018. Net loss for the nine months ended September 30, 2019 was $22.2 million, compared to net loss of $8.2 million for the same period in 2018.
- Research and development expenses for the third quarter of 2019 was $6.5 million, compared to $2.2 million for the same period in 2018. Research and development expense for the nine months ended September 30, 2019 was $16.7 million, compared to $5.4 million for the same period in 2018.
- General and administrative expenses for the three months ended September 30, 2019 were $3.3 million, compared to $1.1 million for the same period in 2018. General and administrative expenses for the nine months ended September 30, 2019 were $7.9 million, compared to $2.9 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 September 30, 2019, Stoke had approximately $233.2 million in cash, cash equivalents and restricted cash, which is anticipated to fund operations into 2023.

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: Stoke's second quarter results; Stoke's expectation about timing and execution of anticipated milestones, including IND submission; the planned initiation of Stoke's BUTTERFLY study and subsequent Phase 1/2 trial, and 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 TANGO 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 set forth in our filings with the Securities and Exchange Commission, including the risks set forth in our quarterly report on Form 10-Q for the quarter and nine months ended September 30, 2019. These forward-looking statements speak only as of the date hereof and Stoke specifically disclaims any obligation to update these forward-looking statements or reasons why actual results might differ, whether as a result of new information, future events or otherwise, except as required by law.

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Financial Tables Follow

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

	September 30, 2019	December 31, 2018
Assets		
Current assets:		
Cash and cash equivalents	$ 233,049	$ 105,399
Prepaid expenses and other current assets	3,255	548
Interest receivable	376	196
Total current assets	$ 236,680	$ 106,143
Restricted cash	205	204
Property and equipment, net	1,942	1,192
Total assets	$ 238,827	$ 107,539
Liabilities and stockholders' equity		
Current liabilities:		
Accounts payable	$ 1,789	$ 1,071
Accrued and other current liabilities	3,127	1,396
Total current liabilities	$ 4,916	$ 2,467
Long term liabilities	228	4
Total liabilities	$ 5,144	$ 2,471
Commitments and contingencies (Note 6)		
Stockholders' equity		
Preferred Stock, par value of $0.0001 per share; 10,000,000 shares authorized, none issued and outstanding as of September 30, 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 September 30, 2019; 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 shares authorized, 32,724,153 and 727,413 shares issued and outstanding as of September 30, 2019 and December 31, 2018, respectively	3	—
Additional paid-in capital	281,555	130,776

Accumulated deficit	(47,875)	(25,710)
Total stockholders' equity	$ 233,683	$ 105,068
Total liabilities and stockholders' equity	$ 238,827	$ 107,539

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

	Three Months Ended September 30,		Nine Months Ended September 30,	
	2019	2018	2019	2018
Revenue	$ —	$ —	$ —	$ —
Operating expenses:				
Research and development	6,518	2,227	16,675	5,367
General and administrative	3,324	1,090	7,935	2,872
Total operating expenses	9,842	3,317	24,610	8,239
Loss from operations	(9,842)	(3,317)	(24,610)	(8,239)
Other income (expense):				
Interest income	1,236	—	2,447	—
Other income (expense), net	2	—	(2)	—
Total other income	1,238	—	2,445	—
Net loss and comprehensive loss	$ (8,604)	$ (3,317)	$ (22,165)	$ (8,239)
Net loss per share attributable to common stockholders, basic and diluted	$ (0.26)	$ (4.75)	$ (1.71)	$ (11.90)
Weighted-average common shares outstanding, basic and diluted	32,707,647	698,970	12,991,672	692,509

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