Stoke Therapeutics Reports Second Quarter Financial Results and Provides Business Updates

August 7, 2024

– FDA removes Partial Clinical Hold as company advances toward a Phase 3 registrational study of zorevunersen (STK-001) in children and adolescents with Dravet syndrome –

– Company to provide an update on Phase 3 registrational plans for zorevunersen in the second half of 2024 –

– Company on track to initiate the Phase 1 study (OSPREY) of STK-002 for the treatment of Autosomal Dominant Optic Atrophy (ADOA) this year –

– As of June 30, 2024, Company had $282.0 million in cash, cash equivalents, and marketable securities –

BEDFORD, Mass.--(BUSINESS WIRE)--Aug. 7, 2024-- Stoke Therapeutics, Inc. (Nasdaq: STOK), a biotechnology company dedicated to restoring protein expression by harnessing the body's potential with RNA medicine, today reported financial results for the second quarter of 2024 and provided business updates including those related to zorevunersen (STK-001), the Company's proprietary antisense oligonucleotide (ASO) which is in development by Stoke as the first potential medicine to address the genetic cause of Dravet syndrome.

"The landmark data announced earlier this year provide strong support for zorevunersen as the first potential disease-modifying medicine for the treatment of Dravet syndrome by showing marked reductions in seizures and improvements in cognition and behavior in a heavily treated patient population," said Edward M. Kaye, M.D., Chief Executive Officer of Stoke Therapeutics. "We continue to receive strong support and hear a great sense of urgency for zorevunersen among the Dravet syndrome community, including families, caregivers and clinicians. We thank the FDA for working with us to remove the Partial Clinical Hold and look forward to continuing our discussions with them and with other global regulatory agencies toward the goal of agreeing on a single, global Phase 3 registrational study design by year-end."

Recent Program Highlights and Upcoming Milestones

- Today, the Company announced that the U.S. Food and Drug Administration (FDA) has removed the Partial Clinical Hold on zorevunersen.
- The Company plans to share previously presented positive data from patients treated in the Phase 1/2a and open label extension (OLE) studies of zorevunersen in children and adolescents with Dravet syndrome at the 15th European Epilepsy Congress (EEC), September 7 – 11, 2024, in Rome, Italy.
- Discussions with global regulatory agencies are underway and the Company is on track to provide a regulatory update on Phase 3 registrational plans for zorevunersen in the second half of 2024.
- The Company is on track to initiate the Phase 1 study (OSPREY) of STK-002 for the treatment of Autosomal Dominant Optic Atrophy (ADOA) this year.

Second Quarter 2024 Financial Results

- As of June 30, 2024, the Company had $282.0 million in cash, cash equivalents, and marketable securities.
- Revenue recognized for upfront license fees and services provided from the License and Collaboration Agreement with Acadia Pharmaceuticals for the three months ended June 30, 2024 was $4.8 million, compared to $(2.5) million for the same period in 2023.
- Net loss for the three months ended June 30, 2024 was $25.7 million, or $0.46 per share, compared to $30.7 million, or $0.69 per share, for the same period in 2023.
- Research and development expenses for the three months ended June 30, 2024 were $21.1 million, compared to $20.6 million for the same period in 2023.
- General and administrative expenses for the three months ended June 30, 2024 were $13.0 million, compared to $10.2 million for the same period in 2023.

Year-to-Date 2024 Financial Results

- Revenue recognized for upfront license fees and services provided from the License and Collaboration Agreement with Acadia Pharmaceuticals for the six months ended June 30, 2024 was $9.0 million, compared to $2.7 million for the same period in 2023.
- Net loss for the six months ended June 30, 2024 was $52.1 million, or $1.02 per share, compared to $53.2 million, or $1.23 per share, for the same period in 2023.
- Research and development expenses for the six months ended June 30, 2024 were $43.5 million, compared to $40.2 million for the same period in 2023.
- General and administrative expenses for the six months ended June 30, 2024 were $23.3 million, compared to $20.4

million for the same period in 2023.

- The increase in operating expenses for the three and six month periods ending June 30, 2024 over the same periods in 2023 primarily relate to increases in costs associated with personnel, third party contracts, consulting, facilities and others associated with development activities for zorevunersen and STK-002, research on additional therapeutics and growing a public corporation.

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 intellectual disability, developmental delays, movement and balance issues, language and speech disturbances, growth defects, sleep abnormalities, disruptions of the autonomic nervous system and mood disorders. 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. There are no approved disease-modifying therapies for people living with Dravet syndrome. One out of 16,000 babies are born with Dravet syndrome, which is not concentrated in a particular geographic area or ethnic group.

About Zorevunersen (STK-001)
Zorevunersen is an investigational new medicine for the treatment of Dravet syndrome currently being evaluated in ongoing clinical trials. Stoke believes that zorevunersen, a proprietary antisense oligonucleotide (ASO), has the potential to be the first disease-modifying therapy to address the genetic cause of Dravet syndrome. Zorevunersen is designed to upregulate NaV1.1 protein expression by leveraging the non-mutant (wild-type) copy of the *SCN1A* gene to restore physiological NaV1.1 levels, thereby reducing both occurrence of seizures and significant non-seizure comorbidities. Zorevunersen has been granted orphan drug designation by the FDA and the EMA, and rare pediatric disease designation by the FDA as a potential new treatment for Dravet syndrome.

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. Severity can vary and the rate of vision loss can be difficult to predict. Roughly half of people with ADOA fail driving standards and up to 46% are registered as legally blind. More than 400 *OPA1* mutations have been reported in people diagnosed with ADOA. Currently there is no approved treatment for people living with ADOA. ADOA affects approximately one in 30,000 people globally with a higher incidence in Denmark of one in 10,000 due to a founder effect.

About STK-002
STK-002 is a proprietary antisense oligonucleotide (ASO) in preclinical development for the treatment of Autosomal Dominant Optic Atrophy (ADOA). Approximately 80% of individuals with ADOA experience symptoms before age 10, typically beginning between the ages of 4 and 6. Stoke believes that STK-002 has the potential to be the first disease-modifying therapy for people living with ADOA. An estimated 65% to 90% of cases are caused by mutations in the *OPA1* gene, most of which lead to a haploinsufficiency resulting in 50% OPA1 protein expression and disease manifestation. STK-002 is designed to upregulate OPA1 protein expression by leveraging the non-mutant (wild-type) copy of the *OPA1* gene to restore OPA1 protein expression with the aim to stop or slow vision loss in patients with ADOA. Stoke has generated preclinical data demonstrating proof-of-mechanism and proof-of-concept for STK-002. STK-002 has been granted orphan drug designation by the FDA as a potential new treatment for ADOA and the company has received authorization of its CTA from the MHRA.

About Stoke Therapeutics
Stoke Therapeutics (Nasdaq: STOK), is a biotechnology company dedicated to restoring protein expression by harnessing the body's potential with RNA medicine. Using Stoke's proprietary TANGO (Targeted Augmentation of Nuclear Gene Output) approach, Stoke is developing antisense oligonucleotides (ASOs) to selectively restore protein levels. Stoke's first compound, zorevunersen (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. Stoke is pursuing the development of STK-002 for the treatment of 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 its 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/.

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: the Company's quarterly results; its future operating results and current or future financial position and liquidity; the ability of zorevunersen (STK-001) to treat the underlying causes of Dravet syndrome and reduce seizures or show improvements in behavior and cognition at the indicated dosing levels or at all; and the timing and expected progress of clinical trials, data readouts, regulatory meetings, regulatory decisions and other presentations. Statements including words such as "expect," "plan," "will," "continue," 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 prove incorrect or do not fully materialize, could cause our results to differ materially from those expressed or implied by such forward-looking statements, including, but not limited to, risks and uncertainties related to: the Company's ability to advance, obtain regulatory approval of, and ultimately commercialize its product candidates, including zorevunersen; the timing of data readouts and interim and final results of preclinical and clinical trials; the receipt and timing of potential regulatory decisions; positive results in a clinical trial may not be replicated in subsequent trials or successes in early stage clinical trials may not be predictive of results in later stage trials; the Company's ability to fund development activities and achieve development goals; the Company's ability to protect its intellectual property; the direct or indirect impact of global business, political and macroeconomic conditions, including inflation, interest rate volatility, cybersecurity events, uncertainty with respect to the federal budget, instability in the global banking system and volatile market conditions, and global events, including public health crises, and ongoing geopolitical conflicts, such as the conflicts in Ukraine and the Middle East; and other risks and uncertainties described under the heading "Risk Factors" in the Company's Annual Report on Form 10-K for the year ended December 31, 2023, its quarterly reports on Form 10-Q, and the other documents it files from time to time with the Securities and Exchange Commission. These forward-looking statements speak only as of the date of this press release, and the Company undertakes no obligation to revise or update any forward-looking statements to reflect events or circumstances after the date hereof.

Financial Tables Follow

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

	June 30, 2024	December 31, 2023
Assets		
Current assets:		
Cash and cash equivalents	$ 193,476	$ 191,442
Marketable securities	88,506	9,952
Prepaid expenses	10,345	11,320
Restricted cash - short-term	75	—
Interest receivable	305	64
Other current assets	4,287	2,561
Total current assets	$ 296,994	$ 215,339
Restricted cash - long-term	494	569
Operating lease right-of-use assets	5,499	6,611
Property and equipment, net	4,770	5,823
Total Assets	$ 307,757	$ 228,342
Liabilities and stockholders' equity		
Current liabilities:		
Accounts payable	$ 3,945	$ 1,695
Accrued and other current liabilities	14,500	13,815
Deferred revenue - current portion	26,051	15,309
Total current liabilities	$ 44,496	$ 30,819
Deferred revenue - net of current portion	16,946	33,074
Other long term liabilities	3,606	4,884
Total long term liabilities	20,552	37,958
Total liabilities	$ 65,048	$ 68,777
Stockholders' equity		
Common stock, par value of $0.0001 per share; 300,000,000 shares authorized, 52,305,641 and 45,918,233 shares issued and outstanding as of June 30, 2024 and December 31, 2023, respectively	5	5
Additional paid-in capital	696,637	561,433
Accumulated other comprehensive loss	(15)	(24)
Accumulated deficit	(453,918)	(401,849)
Total stockholders' equity	$ 242,709	$ 159,565
Total liabilities and stockholders' equity	$ 307,757	$ 228,342

Stoke Therapeutics, Inc. and subsidiary
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,	
	2024	2023	2024	2023
Revenue	$ 4,831	$ (2,481)	$ 9,048	$ 2,671
Operating expenses:				
Research and development	21,136	20,551	43,504	40,182
General and administrative	13,037	10,230	23,258	20,442
Total operating expenses	34,173	30,781	66,762	60,624
Loss from operations	(29,342)	(33,262)	(57,714)	(57,953)
Other income (expense):				
Interest income (expense), net	3,695	2,567	6,121	4,670
Other income (expense), net	(48)	41	(476)	84

Total other income (expense)	3,647	2,608	5,645	4,754
Net loss	$ (25,695)	$ (30,654)	$ (52,069)	$ (53,199)
Net loss per share, basic and diluted	$ (0,46)	$ (0,69)	$ (1,02)	$ (1,23)
Weighted-average common shares outstanding, basic and diluted	55,765,948	44,188,464	51,288,222	43,367,032
Comprehensive loss:				
Net loss	$ (25,695)	$ (30,654)	$ (52,069)	$ (53,199)
Other comprehensive gain (loss):				
Unrealized gain (loss) on marketable securities	(15)	219	9	796
Total other comprehensive gain (loss)	$ (15)	$ 219	$ 9	$ 796
Comprehensive loss	$ (25,710)	$ (30,435)	$ (52,060)	$ (52,403)

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