



Stoke Therapeutics Reports First Quarter Financial Results and Provides Business Updates

May 6, 2024

- Recent data demonstrating reductions in seizure frequency and improvements in cognition and behavior support the potential for STK-001 as a disease-modifying potential medicine for the treatment of Dravet syndrome –
- Company plans to meet with regulatory agencies to discuss registrational study design for STK-001; Update anticipated in the second half of 2024 –
 - As of March 31, 2024, Company had \$178.6 million in cash and cash equivalents –
 - In April, Company strengthened its cash position with \$120.3 million in net proceeds from public follow-on offering –

BEDFORD, Mass.--(BUSINESS WIRE)--May 6, 2024-- [Stoke Therapeutics, Inc.](https://www.stoketherapeutics.com) (Nasdaq: STOK), a biotechnology company dedicated to addressing the underlying cause of severe diseases by upregulating protein expression with RNA-based medicines, today reported financial results for the first quarter of 2024 and provided business updates including those related to 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.

"In the first quarter of 2024, Stoke took a major step forward in our effort to advance the first potential disease-modifying medicine for patients with Dravet syndrome," said Edward M. Kaye, M.D., Chief Executive Officer of Stoke Therapeutics. "Our recent data that showed substantial and durable reductions in seizure frequency and clinically meaningful improvements across multiple measures of cognition and behavior on top of the best available anti-seizure medicines support our belief that we are addressing the root cause of Dravet syndrome. We are working with a sense of urgency to meet with regulatory agencies to discuss our plans for a randomized, controlled registrational study for STK-001 and look forward to providing an update in the second half of 2024."

Recent Program Highlights and Upcoming Milestones

- In March, the Company shared positive new data from 81 patients treated in the Phase 1/2a and OLE studies of STK-001 in children and adolescents with Dravet syndrome. These data showed substantial and durable reductions in seizures and clinically meaningful improvements in multiple measures of cognition and behavior that support the potential for disease modification. Single and multiple doses of STK-001 up to 70mg have been generally well tolerated.
- The Company plans to present these data at the upcoming Seventeenth Eilat Conference on New Antiepileptic Drugs and Devices (EILAT XVII), in Madrid, Spain, May 5-8, 2024. A presentation of these data will also take place at the upcoming American Society of Gene and Cell Therapy (ASGCT) annual meeting in Baltimore, Maryland, May 7-11, 2024.
- The Company plans to meet with regulatory agencies to discuss a randomized, controlled registrational study design with initial doses of 70mg of STK-001 followed by continued dosing at 45mg. An update following those discussions is anticipated in the second half of 2024.
- The Company plans to initiate the Phase 1 study (OSPREY) of STK-002 for the treatment of Autosomal Dominant Optic Atrophy (ADOA) in 2024.
- The Company plans to present initial data from the FALCON natural history study of people living with ADOA at The Association for Research in Vision and Ophthalmology (ARVO) Annual meeting in Seattle, Washington, May 5-9, 2024.
- The Company's collaboration with Acadia Pharmaceuticals to discover, develop and commercialize novel RNA-based medicines for the potential treatment of severe and rare genetic neurodevelopmental diseases of the central nervous system (CNS) is ongoing. The collaboration includes Rett syndrome (MECP2), SYNGAP1, and an undisclosed neurodevelopmental target of mutual interest.

Additional Corporate Highlights

- On April 2, 2024, the Company completed an underwritten public offering of common stock and pre-funded warrants that resulted in net proceeds of \$120.3 million after deducting underwriting discounts and commissions.
- The Company expanded and strengthened its management team with the appointments of Jason Hoitt as Chief Commercial Officer and Thomas (Tommy) Leggett as Chief Financial Officer.

First Quarter 2024 Financial Results

- As of March 31, 2024, the Company had \$178.6 million in cash and cash equivalents.
- Revenue recognized for upfront license fees and services provided from a License and Collaboration Agreement with Acadia Pharmaceuticals for the three months ended March 31, 2024 was \$4.2 million, compared to \$5.2 million, for the same period in 2023.
- Net loss for the three months ended March 31, 2024 was \$26.4 million, or \$0.57 per share, compared to \$22.5 million, or

\$0.53 per share, for the same period in 2023.

- Research and development expenses for the three months ended March 31, 2024 were \$22.4 million, compared to \$19.6 million for the same period in 2023.
- General and administrative expenses for the three months ended March 31, 2024 were \$10.2 million, compared to \$10.2 million for the same period in 2023.
- The increase in operating expenses for the three months ended March 31, 2024 compared to the same period in 2023 primarily relate to increases in costs associated with personnel, third party contracts, consulting, facilities and others associated with development activities for STK-001 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 STK-001

STK-001 is an investigational new medicine for the treatment of Dravet syndrome currently being evaluated in ongoing clinical trials. 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 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. STK-001 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 addressing the underlying cause of severe diseases by upregulating protein expression with RNA-based medicines. 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, 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 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 STK-001; 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)

	March 31, 2024	December 31, 2023
Assets		
Current assets:		
Cash and cash equivalents	\$ 178,581	\$ 191,442
Marketable securities	—	9,952
Prepaid expenses	10,722	11,320
Other current assets	3,559	2,561
Deferred financing costs	402	—
Interest receivable	11	64
Total current assets	<u>\$ 193,275</u>	<u>\$ 215,339</u>
Restricted cash	569	569
Operating lease right-of-use assets	6,060	6,611
Property and equipment, net	5,278	5,823
Total assets	<u>\$ 205,182</u>	<u>\$ 228,342</u>
Liabilities and stockholders' equity		
Current liabilities:		
Accounts payable	\$ 2,102	\$ 1,695
Accrued and other current liabilities	12,570	13,815
Deferred revenue - current portion	20,918	15,309
Total current liabilities	<u>\$ 35,590</u>	<u>\$ 30,819</u>
Deferred revenue - net of current portion	25,042	33,074
Other long term liabilities	4,208	4,884
Total long term liabilities	<u>29,250</u>	<u>37,958</u>
Total liabilities	<u>\$ 64,840</u>	<u>\$ 68,777</u>
Commitments and contingencies		
Stockholders' equity		
Common stock, par value of \$0.0001 per share; 300,000,000 shares authorized, 46,498,077 and 45,918,233 shares issued and outstanding as of March 31, 2024 and December 31, 2023, respectively	5	5
Additional paid-in capital	568,560	561,433
Accumulated other comprehensive loss	—	(24)
Accumulated deficit	(428,223)	(401,849)
Total stockholders' equity	<u>\$ 140,342</u>	<u>\$ 159,565</u>
Total liabilities and stockholders' equity	<u>\$ 205,182</u>	<u>\$ 228,342</u>

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

	Three Months Ended March 31,	
	2024	2023
Revenue	\$ 4,216	\$ 5,152
Operating expenses:		
Research and development	22,368	19,631
General and administrative	10,220	10,211
Total operating expenses	<u>32,588</u>	<u>29,842</u>

Loss from operations	(28,372)	(24,690)
Other income (expense):		
Interest income (expense), net	2,426	2,103
Other income (expense), net	(428)	42
Total other income (expense)	1,998	2,145
Net loss	\$ (26,374)	\$ (22,545)
Net loss per share, basic and diluted	\$ (0.57)	\$ (0.53)
Weighted-average common shares outstanding, basic and diluted	46,246,889	42,536,474
Comprehensive loss:		
Net loss	\$ (26,374)	\$ (22,545)
Other comprehensive gain (loss):		
Unrealized gain (loss) on marketable securities	24	577
Total other comprehensive gain	\$ 24	\$ 577
Comprehensive loss	\$ (26,350)	\$ (21,968)

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