



## Stoke Therapeutics Reports Third Quarter Financial Results and Provides Business Updates

November 5, 2024

– Company plans to provide seizure as well as cognition and behavior data from all patients treated with initial 70mg doses followed by 45mg maintenance dosing in studies of zorevunersen by year-end –

– Discussions with FDA and global regulatory agencies related to a single global Phase 3 study of zorevunersen continue to progress; Company to provide an update by year-end –

– As of September 30, 2024, Company had \$269.2 million in cash, cash equivalents, and marketable securities –

BEDFORD, Mass.--(BUSINESS WIRE)--Nov. 5, 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 third quarter of 2024 and provided business updates including those related to zorevunersen, 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.

"We are headed into a busy year-end as we prepare to share new data which is representative of our proposed Phase 3 dosing regimen and complete our regulatory discussions toward alignment on that study design," said Edward M. Kaye, M.D., Chief Executive Officer of Stoke Therapeutics. "Key to our regulatory discussions are the assessments of behavior and cognition in patients with Dravet syndrome. In our studies of zorevunersen, we have demonstrated substantial and sustained effects across multiple measures of disease, starting within the first year of treatment. These data give us confidence in our Phase 3 planning and the potential for zorevunersen to go beyond seizure management by addressing the root cause of the disease."

The Company announced today that it has submitted abstracts for presentation at the American Epilepsy Society meeting taking place December 6-10, in Los Angeles, California. Included among these planned presentations are new data from all patients treated in the clinical studies with initial 70mg doses followed by 45mg maintenance dosing in studies of zorevunersen. A Phase 3 regimen of two or three loading doses of 70mg followed by maintenance doses of 45mg is currently under discussion with global regulatory agencies. As the Company continues to focus on zorevunersen as its lead program, it will delay the start of the Phase 1 study of STK-002, its clinical candidate for the treatment of autosomal dominant optic atrophy (ADOA).

### Recent Program Highlights and Upcoming Milestones

- In August, the Company announced that the U.S. Food and Drug Administration (FDA) has removed the Partial Clinical Hold on zorevunersen.
- In September, the Company shared data from the Phase 1/2a and open-label extension (OLE) studies of zorevunersen in children and adolescents with Dravet syndrome at the 15<sup>th</sup> European Epilepsy Congress (EEC).
- Company management will present at the Jefferies London Healthcare Conference on Wednesday, November 20, at 6:30am EST (11:30am GMT). A live webcast of the presentation will be available on the Investors & News section of Stoke's website.
- The Company expects to present data at the American Epilepsy Society (AES) 2024 Annual Meeting, pending acceptance of the abstracts.
- The Company plans to provide an update on Phase 3 registrational plans for zorevunersen by year-end.

### Third Quarter 2024 Financial Results

- As of September 30, 2024, the Company had \$269.2 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 September 30, 2024 was \$4.9 million, compared to \$3.3 million for the same period in 2023.
- Net loss for the three months ended September 30, 2024 was \$26.4 million, or \$0.47 per share, compared to \$24.5 million, or \$0.55 per share, for the same period in 2023.
- Research and development expenses for the three months ended September 30, 2024 were \$22.2 million, compared to \$20.3 million for the same period in 2023.
- General and administrative expenses for the three months ended September 30, 2024 were \$12.7 million, compared to \$10.3 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 nine months ended September 30, 2024 was \$13.9 million, compared to \$6.0 million for

the same period in 2023.

- Net loss for the nine months ended September 30, 2024 was \$78.5 million, or \$1.48 per share, compared to \$77.7 million, or \$1.78 per share, for the same period in 2023.
- Research and development expenses for the nine months ended September 30, 2024 were \$65.7 million, compared to \$60.5 million for the same period in 2023.
- General and administrative expenses for the nine months ended September 30, 2024 were \$36.0 million, compared to \$30.7 million for the same period in 2023.
- The increase in operating expenses for the three and nine month periods ending September 30, 2024 over the same periods in 2023 primarily relates to increases in costs associated with personnel, third party contracts, consulting, facilities and other costs 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  $Na_v1.1$  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. 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 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 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, including expectations regarding its collaboration with Acadia Pharmaceuticals; 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)**

	September 30, 2024	December 31, 2023
<b>Assets</b>		
Current assets:		
Cash and cash equivalents	\$ 150,016	\$ 191,442
Marketable securities - current	89,184	9,952
Prepaid expenses	9,837	11,320
Restricted cash - current	75	—
Interest receivable	691	64
Other current assets	3,809	2,561
Total current assets	\$ 253,612	\$ 215,339
Marketable securities - long-term	29,952	—
Restricted cash - long-term	494	569
Operating lease right-of-use assets	4,928	6,611
Property and equipment, net	4,333	5,823
Total assets	\$ 293,319	\$ 228,342
<b>Liabilities and stockholders' equity</b>		
Current liabilities:		
Accounts payable	\$ 2,485	\$ 1,695
Accrued and other current liabilities	15,768	13,815
Deferred revenue - current portion	31,612	15,309
Total current liabilities	\$ 49,865	\$ 30,819
Deferred revenue - net of current portion	8,291	33,074
Other long term liabilities	3,050	4,884
Total long term liabilities	11,341	37,958
Total liabilities	\$ 61,206	\$ 68,777
Stockholders' equity		
Common stock, par value of \$0.0001 per share; 300,000,000 shares authorized, 52,941,191 and 45,918,233 shares issued and outstanding as of September 30, 2024 and December 31, 2023, respectively	5	5
Additional paid-in capital	712,290	561,433
Accumulated other comprehensive income (loss)	166	(24)
Accumulated deficit	(480,348)	(401,849)
Total stockholders' equity	\$ 232,113	\$ 159,565
Total liabilities and stockholders' equity	\$ 293,319	\$ 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 September 30,		Nine Months Ended September 30,	
	2024	2023	2024	2023
Revenue	\$ 4,894	\$ 3,308	\$ 13,941	\$ 5,978
Operating expenses:				
Research and development	22,205	20,271	65,710	60,453
General and administrative	12,692	10,271	35,950	30,712
Total operating expenses	34,897	30,542	101,660	91,165
Loss from operations	(30,003)	(27,234)	(87,719)	(85,187)
Other income (expense):				
Interest income (expense), net	3,545	2,651	9,668	7,321
Other income (expense), net	28	41	(448)	125
Total other income (expense)	3,573	2,692	9,220	7,446
Net loss	\$ (26,430)	\$ (24,542)	\$ (78,499)	\$ (77,741)
Net loss per share, basic and diluted	\$ (0.47)	\$ (0.55)	\$ (1.48)	\$ (1.78)
Weighted-average common shares outstanding, basic and diluted	56,341,074	44,266,017	52,991,015	43,669,987

Comprehensive loss:				
Net loss	\$ (26,430)	\$ (24,542)	\$ (78,499)	\$ (77,741)
Other comprehensive gain:				
Unrealized gain on marketable securities	181	232	190	1,028
Total other comprehensive gain	\$ 181	\$ 232	\$ 190	\$ 1,028
Comprehensive loss	\$ (26,249)	\$ (24,310)	\$ (78,309)	\$ (76,713)

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