Stoke Therapeutics Reports Fourth Quarter and Full Year 2024 Financial Results and Provides Business Updates

March 18, 2025

– Phase 3 EMPEROR study of zorevunersen, a first-in-class potential disease-modifying medicine for Dravet syndrome, on track to initiate in 2Q 2025 –

– As of December 31, 2024, the Company had $246.7 million in cash, cash equivalents, and marketable securities; together with the $165 million upfront and eligible proceeds from Biogen collaboration anticipated to fund operations to mid-2028 –

BEDFORD, Mass.--(BUSINESS WIRE)--Mar. 18, 2025-- Stoke Therapeutics, Inc. (Nasdaq: STOK) is a biotechnology company dedicated to restoring protein expression by harnessing the body's potential with RNA medicine and has a lead investigational medicine, zorevunersen, in development as a first-in-class potential disease-modifying treatment for Dravet syndrome. The Company today reported financial results for the full year ended December 31, 2024 and provided business updates.

"Recent milestones – including Breakthrough Therapy Designation, positive data supporting our Phase 3 dosing regimen and global regulatory alignment – have catalyzed the Dravet community and put us on track to initiate EMPEROR in the second quarter," said Edward M. Kaye, M.D., Chief Executive Officer of Stoke Therapeutics. "The potential of zorevunersen to change the course of Dravet syndrome by addressing its underlying cause is becoming increasingly recognized. Our recent collaboration with Biogen brings complementary expertise in neurology and the global commercialization of high-value medicines for rare genetic diseases – expertise that will help us deliver faster for patients and maximize value. With a strong financial position, we are well-capitalized to advance through Phase 3 and prepare for launch readiness."

Recent Program Highlights and Additional Corporate Updates

- Separately today, the Company announced that Dr. Edward M. Kaye has decided to step down as Chief Executive Officer of Stoke Therapeutics. After seven years of successful leadership and taking the company from a startup to a late-stage clinical development company, Dr. Kaye will transition to an advisory role and continue to serve as a Director.
- In February, the Company entered into a collaboration with Biogen to develop and commercialize zorevunersen for the treatment of Dravet syndrome. The Company retains exclusive rights for zorevunersen in the United States, Canada, and Mexico; Biogen receives exclusive rest of world commercialization rights.
- In January, the Company announced plans to initiate a global Phase 3 registrational study of zorevunersen (EMPEROR) following successful alignment with regulatory agencies in the United States, Europe, and Japan.
- In December, the Company shared new positive data from the open-label extension (OLE) studies of zorevunersen in children and adolescents with Dravet syndrome at American Epilepsy Society (AES) 2024 Annual Meeting.
- In December, the Company announced that the U.S. Food and Drug Administration (FDA) granted Breakthrough Therapy Designation for zorevunersen for the treatment of Dravet syndrome with a confirmed mutation, not associated with gain-of-function, in the *SCN1A* gene.

Year End 2024 Financial Results

- As of December 31, 2024, the Company had $246.7 million in cash, cash equivalents, and marketable securities, which, along with the $165 million upfront payment received in March 2025 and other eligible cash flows from the Biogen collaboration, we anticipate will fund operations to mid-2028.
- Revenue recognized for upfront license fees and services provided from the License and Collaboration Agreement with Acadia Pharmaceuticals for the year ended December 31, 2024 was $36.6 million, compared to $8.8 million, for the year ended December 31, 2023.
- Net loss for the year ended December 31, 2024 was $89.0 million, or $1.65 per share, compared to $104.7 million, or $2.38 per share for 2023.
- Research and development expenses for the year ended December 31, 2024 were $89.1 million, compared to $82.2 million for 2023.
- General and administrative expenses for the year ended December 31, 2024 were $48.8 million, compared to $41.3 million for 2023.
- The increase in operating expenses for the year ended December 31, 2024 compared to the same period 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.

Fourth Quarter 2024 Financial Results

- Revenue recognized for upfront license fees and services provided from the License and Collaboration Agreement with Acadia Pharmaceuticals for the three months ended December 31, 2024 was $22.6 million, compared to $2.8 million for the same period in 2023.
- Net loss for the three months ended December 31, 2024 was $10.5 million, or $0.18 per share, compared to $27.0 million, or $0.60 per share, for the same period in 2023.
- Research and development expenses for the three months ended December 31, 2024 were $23.4 million, compared to $21.8 million for the same period in 2023.
- General and administrative expenses for the three months ended December 31, 2024 were $12.8 million, compared to $10.6 million for the same period in 2023.
- The increase in operating expenses for the three months ended December 31, 2024 compared to the same period 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 developmental and epileptic encephalopathy characterized by severe, recurrent seizures as well as significant cognitive and behavioral impairments. Most cases of Dravet are caused by mutations in one copy of the *SCN1A* gene, leading to insufficient levels of NaV1.1 protein in neuronal cells in the brain. More than 90 percent of patients continue to experience seizures despite treatment with the best available anti-seizure medicines. Complications of the disease often contribute to a poor quality of life for patients and their caregivers. Developmental and cognitive impairments 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. 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 occurs globally and is not concentrated in a particular geographic area or ethnic group. Currently, it is estimated that up to 38,000 people are living with Dravet syndrome in the U.S., UK, EU-4 and Japan.

About Zorevunersen
Zorevunersen is an investigational antisense oligonucleotide that is designed to treat the underlying cause of Dravet syndrome by increasing NaV1.1 protein production in brain cells from the non-mutated (wild-type) copy of the *SCN1A* gene. This highly differentiated mechanism of action aims to reduce seizure frequency beyond what has been achieved with anti-seizure medicines and to improve neurodevelopment, cognition, and behavior. Zorevunersen has demonstrated the potential for disease modification and has been granted orphan drug designation by the FDA and the EMA. The FDA has also granted zorevunersen rare pediatric disease designation and Breakthrough Therapy Designation for the treatment of Dravet syndrome with a confirmed mutation not associated with gain-of-function, in the *SCN1A* gene.

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 naturally-occurring protein levels. Stoke's first medicine in development, zorevunersen, has demonstrated the potential for disease modification in patients with Dravet syndrome and is expected to enter Phase 3 development in 2025. Stoke's initial focus are diseases of the central nervous system and the eye that are caused by a loss of ~50% of normal protein levels (haploinsufficiency). Proof of concept has been demonstrated in other organs, tissues, and systems, supporting broad potential for the Company's 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 receipt of potential cashflows under the collaboration with Biogen; the design, timing and results of the Phase 3 EMPEROR study; the timing and expected progress of regulatory filings and regulatory decisions; the ability of zorevunersen 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; the Company's cash runway; and the expectations regarding the collaborations. Statements including words such as "anticipate," "expect," "plan," "will," or "may" 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 the Company's 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 and ultimately commercialize its product candidates; that if Biogen were to breach or terminate the collaboration, the Company would not obtain the anticipated financial or other benefits; the possibility that the Company and Biogen may not be successful in their development of zorevunersen and that, even if successful, they may be unable to successfully commercialize zorevunersen; 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 protect its intellectual property; the Company's ability to fund development activities and achieve development goals through mid-2028; and the 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, 2024, its quarterly reports on Form 10-Q, and the other documents it files 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)

	As of December 31,	
	2024	2023
Assets		
Current assets:		
Cash and cash equivalents	$ 127,983	$ 191,442
Marketable securities - short term	88,916	9,952
Prepaid expenses	11,117	11,320
Other current assets	3,965	2,561
Restricted cash - short term	75	—
Interest receivable	700	64
Total current assets	$ 232,756	$ 215,339
Restricted cash - long term	721	569
Operating lease right-of-use assets	4,345	6,611
Marketable securities - long term	29,824	—
Property and equipment, net	3,909	5,823
Total assets	$ 271,555	$ 228,342
Liabilities and stockholders' equity		
Current liabilities:		
Accounts payable	$ 2,498	$ 1,695
Accrued and other current liabilities	18,567	13,815
Deferred revenue - current portion	18,991	15,309
Total current liabilities	$ 40,056	$ 30,819
Deferred revenue - net of current portion	—	33,074
Other long term liabilities	2,478	4,884
Total long term liabilities	$ 2,478	$ 37,958
Total liabilities	$ 42,534	$ 68,777
Commitments and contingencies		
Stockholders' equity		
Common stock, par value of $0.0001 per share; 300,000,000 shares authorized, 54,032,826 and 45,918,233 shares issued and outstanding as of December 31, 2024 and 2023, respectively	5	5
Additional paid-in capital	719,997	561,433
Accumulated other comprehensive loss	(151)	(24)
Accumulated deficit	(490,830)	(401,849)
Total stockholders' equity	$ 229,021	$ 159,565
Total liabilities and stockholders' equity	$ 271,555	$ 228,342

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

	Three Months Ended December 31, (unaudited)		Year Ended December 31,	
	2024	2023	2024	2023
Revenue	$ 22,614	$ 2,801	$ 36,555	$ 8,780
Operating expenses:				
Research and development	23,424	21,778	89,133	82,231
General and administrative	12,844	10,610	48,794	41,322
Total operating expenses	36,268	32,388	137,927	123,553
Loss from operations	(13,654)	(29,587)	(101,372)	(114,773)
Other income (expense):				
Interest income (expense), net	2,971	2,587	12,638	9,908
Other income (expense), net	200	42	(247)	166
Total other income (expense)	3,171	2,629	12,391	10,074
Net loss	$ (10,483)	$ (26,958)	$ (88,981)	$ (104,699)
Net loss per share—basic and diluted	$ (0.18)	$ (0.60)	$ (1.65)	$ (2.38)
Weighted average common shares outstanding—basic and diluted	57,029,296	44,958,894	54,008,883	43,994,862

Comprehensive loss:				
Net loss	$ (10,483)	$ (26,958)	$ (88,981)	$ (104,699)
Other comprehensive loss:				
Unrealized gain (loss) on marketable securities	(317)	122	(127)	1,151
Total other comprehensive gain (loss)	$ (317)	$ 122	$ (127)	$ 1,151
Comprehensive loss	$ (10,800)	$ (26,836)	$ (89,108)	$ (103,548)

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