



Stoke Therapeutics Reports First Quarter 2025 Financial Results and Provides Business Updates

May 13, 2025

- Regulatory alignment achieved for Phase 3 EMPEROR study of zorevunersen, a potential first-in-class disease-modifying medicine for Dravet syndrome; Study start anticipated in 2Q 2025 –
- Zorevunersen collaboration with Biogen brings leading capabilities for commercializing high value, disease-modifying medicines for rare genetic diseases; Stoke retains full rights in the United States, Canada, and Mexico –
- As of March 31, 2025, the Company had \$380.3 million in cash, cash equivalents, and marketable securities, anticipated to fund operations beyond 2H 2027 Phase 3 data and into launch readiness projected to mid-2028 –

BEDFORD, Mass.--(BUSINESS WIRE)--May 13, 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 first quarter ended March 31, 2025, and provided business updates.

"The first quarter was defined by strong execution as we advance zorevunersen into Phase 3 development for Dravet syndrome," said Ian F. Smith, Interim Chief Executive Officer and Director of Stoke Therapeutics. "With regulatory alignment on a global Phase 3 study, a strategic collaboration with Biogen, and a balance sheet that takes the company to launch readiness, we are well-positioned to deliver the first disease-modifying therapy for Dravet syndrome to patients worldwide who need it while also continuing to invest in our pipeline."

Recent Program Highlights and Upcoming Milestones

- The Company plans to initiate EMPEROR in the second quarter of 2025 with an expected data readout in the second half of 2027, which is anticipated to support global regulatory filings.
- In February, the Company entered into a collaboration with Biogen to develop and commercialize zorevunersen for the treatment of Dravet syndrome for which Stoke received \$165 million upfront. The parties will share external clinical development costs for zorevunersen (30 percent Biogen; 70 percent Stoke). Additionally, Stoke may receive up to \$385 million in development and commercial milestone payments and be eligible to receive tiered royalties ranging from low double digits to high teens on potential net sales in the Biogen territory. Stoke retains exclusive rights for zorevunersen in the United States, Canada, and Mexico. Biogen receives exclusive rest of world commercialization rights. (For full details, see the [press release](#)).
- In January, the Company announced plans to initiate a global Phase 3 registrational study of zorevunersen (EMPEROR) for the treatment of Dravet syndrome following successful alignment with the U.S. Food and Drug Administration (FDA), European Medicines Agency (EMA) and Japan's Pharmaceuticals and Medical Devices Agency (PMDA). The study design is based on positive clinical data from Phase 1/2a and open label extension studies that showed continuing improvements in cognition and behavior in addition to substantial and durable reductions in major motor seizure frequency. The study will evaluate two loading doses of 70mg followed by two maintenance doses of 45mg over 52 weeks compared to sham in children and adolescents ages 2 to <18 with Dravet syndrome. The primary endpoint will be reduction in major motor seizure frequency. Key secondary endpoints will include improvements in cognition and behavior as measured primarily by Vineland-3. (For full details, see the [press release](#)).

First Quarter 2025 Financial Results

- As of March 31, 2025, the Company had \$380.3 million in cash, cash equivalents, and marketable securities, anticipated to 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 three months ended March 31, 2025, was \$6.1 million, compared to \$4.2 million, for the same period in 2024.
- Revenue recognized from the License and Collaboration Agreement with Biogen for the three months ended March 31, 2025, was \$152.4 million, there was no revenue for the same period in 2024.
- Net income for the three months ended March 31, 2025, was \$112.9 million, or \$1.90 per diluted share, compared to a net loss of \$26.4 million, or \$0.57 per share for the same period in 2024.
- Research and development expenses for the three months ended March 31, 2025, were \$32.7 million, compared to \$22.4 million for the same period in 2024. This includes a one-time \$8.2 million sublicense fee associated with the Biogen agreement.

- General and administrative expenses for the three months ended March 31, 2025, were \$14.7 million, compared to \$10.2 million for the same period in 2024 due to an increase in personnel and launch readiness expense.

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 Company's quarterly results; its future operating results and current or future financial position and liquidity; 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)

	March 31, 2025	December 31, 2024
Assets		
Current assets:		
Cash and cash equivalents	\$ 274,817	\$ 127,983
Marketable securities - current	82,507	88,916
Prepaid expenses	12,694	11,117
Restricted cash - current	75	75
Interest receivable	606	700
Other current assets	5,037	3,965
Total current assets	\$ 375,736	\$ 232,756

Marketable securities - long-term	22,989	29,824
Restricted cash - long-term	721	721
Operating lease right-of-use assets	3,831	4,345
Property and equipment, net	3,611	3,909
Total assets	<u>\$ 406,888</u>	<u>\$ 271,555</u>
Liabilities and stockholders' equity		
Current liabilities:		
Accounts payable	\$ 2,186	\$ 2,498
Accrued and other current liabilities	24,335	18,567
Deferred revenue - current portion	18,164	18,991
Total current liabilities	<u>\$ 44,685</u>	<u>\$ 40,056</u>
Deferred revenue - net of current portion	10,255	—
Other long term liabilities	1,874	2,478
Total long term liabilities	<u>12,129</u>	<u>2,478</u>
Total liabilities	<u>\$ 56,814</u>	<u>\$ 42,534</u>
Stockholders' equity		
Common stock, par value of \$0.0001 per share; 300,000,000 shares authorized, 54,595,714 and 54,032,826 shares issued and outstanding as of March 31, 2025 and December 31, 2024, respectively	5	5
Additional paid-in capital	728,124	719,997
Accumulated other comprehensive loss	(104)	(151)
Accumulated deficit	(377,951)	(490,830)
Total stockholders' equity	<u>\$ 350,074</u>	<u>\$ 229,021</u>
Total liabilities and stockholders' equity	<u>\$ 406,888</u>	<u>\$ 271,555</u>

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

	Three Months Ended March 31,	
	2025	2024
Revenue	<u>\$ 158,569</u>	<u>\$ 4,216</u>
Operating expenses:		
Research and development	32,676	22,368
General and administrative	14,653	10,220
Total operating expenses	<u>47,329</u>	<u>32,588</u>
Income (loss) from operations	<u>111,240</u>	<u>(28,372)</u>
Other income (expense):		
Interest income (expense), net	2,889	2,426
Other income (expense), net	28	(428)
Total other income (expense)	<u>2,917</u>	<u>1,998</u>
Income (loss) before income taxes	<u>\$ 114,157</u>	<u>\$ (26,374)</u>
Provision for income taxes	1,278	—
Net income (loss)	<u>\$ 112,879</u>	<u>\$ (26,374)</u>
Net income (loss) per share:		
Basic	\$ 1.95	\$ (0.57)
Diluted	1.90	(0.57)
Weighted-average common shares outstanding:		
Basic	57,862,674	46,246,889
Diluted	61,123,404	46,246,889
Comprehensive income (loss):		
Net income (loss)	<u>\$ 112,879</u>	<u>\$ (26,374)</u>
Other comprehensive gain:		
Unrealized gain on marketable securities	47	24
Total other comprehensive gain	<u>\$ 47</u>	<u>\$ 24</u>
Comprehensive income (loss)	<u>\$ 112,926</u>	<u>\$ (26,350)</u>

View source version on [businesswire.com](https://www.businesswire.com/news/home/20250513794657/en/): <https://www.businesswire.com/news/home/20250513794657/en/>

Stoke Media & Investor Contacts:

Dawn Kalmar

Chief Communications Officer

dkalmar@stoketherapeutics.com

781-303-8302

Doug Snow

Director, Communications & Investor Relations

IR@stoketherapeutics.com

508-642-6485

Source: Stoke Therapeutics, Inc.