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

August 12, 2025

– First patient dosed in the Phase 3 EMPEROR study of zorevunersen in patients with Dravet syndrome –

– New 3-year zorevunersen OLE data provide additional support for disease modification: continuing and durable reductions in seizures and improvements in cognition and behavior and generally well tolerated –

– Phase 1 study of STK-002 initiated in patients with Autosomal Dominant Optic Atrophy (ADOA), the most common inherited optic nerve disorder –

– As of June 30, 2025, the Company had $355.0 million in cash, cash equivalents, and marketable securities, anticipated to fund operations to mid-2028 and into launch readiness –

– Webcast and conference call for analysts and investors at 4:30PM Eastern Time today –

BEDFORD, Mass.--(BUSINESS WIRE)--Aug. 12, 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 second quarter ended June 30, 2025 and provided business updates.

"This quarter was defined by strong execution that is driving momentum across our business," said Ian F. Smith, Interim Chief Executive Officer and Director of Stoke Therapeutics. "Our Phase 1/2 and open-label extension studies have provided a strong foundational understanding of zorevunersen and support the EMPEROR Phase 3 registrational study design. We continue to generate long-term data that are helping us appreciate the disease modifying potential of zorevunersen. At the same time, we see growing awareness of Dravet syndrome as a severe neurodevelopmental disease, which is bringing attention to our work and a high degree of interest in zorevunersen."

Smith continued, "Beyond Dravet, we have initiated clinical development in a second disease area, advancing STK-002 into a Phase 1 study in patients with Autosomal Dominant Optic Atrophy. Without any treatments approved for ADOA, patients are at risk of progressive loss of sight caused primarily by insufficient OPA1 protein levels. Our pre-clinical data support the potential for STK-002 to restore naturally-occurring protein expression to maintain or improve vision in these patients. We look forward to continuing to expand our approach into new disease areas where we believe we can deliver first-in-class, disease modifying medicines for severe genetic diseases."

Recent Program Highlights

- Yesterday, the Company announced that the first patient has been dosed in the global Phase 3 EMPEROR study of zorevunersen for the treatment of Dravet syndrome. Sites have been initiated in the U.S., UK, Japan and are planned for Europe.
- Today, the Company announced new positive findings from the long-term open-label extension (OLE) studies of zorevunersen in children and adolescents with Dravet syndrome. Substantial and durable reductions in convulsive seizure frequency on top of standard-of-care medicines were observed through three years of zorevunersen treatment. The data also demonstrate continued improvements in cognition and behavior during the 3-year OLE period beyond the initial 9 months of treatment in the Phase 1/2 studies.
- Today, the Company announced the Phase 1 study (OSPREY) of STK-002 in patients with Autosomal Dominant Optic Atrophy (ADOA) is now underway.
- In July, the Company presented data at the European Paediatric Neurology Society (EPNS) Congress from an analysis that evaluated the potential effects on cognition and behavior of a dosing regimen similar to the one now being evaluated in Phase 3. (For full details, see the press release).

Upcoming Anticipated Milestones

- The Company plans to present additional data from the zorevunersen clinical development program at upcoming medical congresses in 2025.
- Lead optimization is underway to identify a clinical candidate for the treatment of SYNGAP-1 in 2026. SYNGAP-1 is a severe and rare genetic neurodevelopmental disease.

Second Quarter 2025 Financial Results

- As of June 30, 2025, the Company had $355.0 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 June 30, 2025 was $10.6 million, compared to $4.8 million, for the same period in 2024.
- Revenue recognized from the License and Collaboration Agreement with Biogen for the three months ended June 30, 2025 was $3.2 million. There was no revenue for the same period in 2024.
- Net loss for the three months ended June 30, 2025 was $23.5 million, or $0.40 per share, compared to a net loss of $25.7 million, or $0.46 per share for the same period in 2024.
- Research and development expenses for the three months ended June 30, 2025 were $25.9 million, compared to $21.1 million for the same period in 2024.
- General and administrative expenses for the three months ended June 30, 2025 were $15.3 million, compared to $13.0 million for the same period in 2024.

Year-to-Date 2025 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, 2025 was $16.8 million, compared to $9.0 million for the same period in 2024.
- Revenue recognized from the License and Collaboration Agreement with Biogen for the six months ended June 30, 2025 was $155.6 million. There was no revenue for the same period in 2024.
- Net income for the six months ended June 30, 2025 was $89.4 million, or $1.50 per diluted share, compared to a net loss of $52.1 million, or $1.02 per share, for the same period in 2024.
- Research and development expenses for the six months ended June 30, 2025 were $58.5 million, compared to $43.5 million for the same period in 2024.
- General and administrative expenses for the six months ended June 30, 2025 were $29.9 million, compared to $23.3 million for the same period in 2024.
- The increase in operating expenses for the three and six month periods ending June 30, 2025 over the same periods in 2024 primarily relate to increases in costs associated with an increase in personnel and launch readiness expense.

Stoke Webcast and Conference Call for Analysts and Investors
Stoke management will host a webcast and conference call for analysts and investors on Tuesday, August 12, 2025, at 4:30pm Eastern Time. The webcast will be available on the Investors & News section of Stoke's website at https://investor.stoketherapeutics.com/. Research analysts who plan to join the call and participate in the Q&A session may register here to receive the dial-in details and a unique PIN. All other participants are invited to access the listen-only webcast by clicking here. A replay of the webcast will be archived and available for at least 90 days following the event.

About Dravet Syndrome
Dravet syndrome is a severe developmental and epileptic encephalopathy (DEE) 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. (~16,000), UK, EU-4 and Japan.[1]

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. Stoke has a strategic collaboration with Biogen to develop and commercialize zorevunersen for Dravet syndrome. Under the collaboration, Stoke retains exclusive rights for zorevunersen in the United States, Canada, and Mexico; Biogen receives exclusive rest of world commercialization rights.

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 different *OPA1* mutations have been reported in people diagnosed 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. Currently there is no approved treatment for people living with ADOA.

About STK-002
STK-002 is a proprietary antisense oligonucleotide (ASO) in clinical development for the treatment of Autosomal Dominant Optic Atrophy (ADOA). ADOA causes progressive and irreversible vision loss in both eyes starting in the first decade of life. 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 maintain or improve vision 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. A Phase 1 study (OSPREY) of STK-002 in patients with ADOA is now underway.

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 currently being evaluated in a Phase 3 study. 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 Stoke's proprietary approach. Stoke is headquartered in Bedford, 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 and cash runway; its future operating results and current or future financial position and liquidity; 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 design, timing and results of clinical studies, data readouts, regulatory decisions and other presentations for zorevunersen and STK-002; the ability of STK-002 to treat the underlying causes of Autosomal Dominant Optic Atrophy (ADOA) and maintain or improve vision; our expectations, plans, aspirations and goals, including those related to the potential of zorevunersen and our collaborations with Biogen and Acadia. 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.

Reference:

1. Based on Stoke Therapeutics' preliminary estimates, which scaled annual incidence to prevalence using country-specific live birth rates over the past 85 years and adjusted for Dravet-specific mortality. The estimate is based on incidence rates published by Wu et al., Pediatrics, 2015.

Financial Tables Follow

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

	June 30, 2025	December 31, 2024
Assets		
Current assets:		
Cash and cash equivalents	$ 101,472	$ 127,983
Marketable securities - current	146,236	88,916
Prepaid expenses	13,694	11,117
Restricted cash - current	75	75
Interest receivable	1,622	700
Other current assets	6,871	3,965
Total current assets	$ 269,970	$ 232,756
Marketable securities - long-term	107,256	29,824
Restricted cash - long-term	721	721
Operating lease right-of-use assets	3,218	4,345
Property and equipment, net	3,343	3,909
Total assets	$ 384,508	$ 271,555
Liabilities and stockholders' equity		
Current liabilities:		
Accounts payable	$ 4,313	$ 2,498
Accrued and other current liabilities	25,616	18,567
Deferred revenue - current portion	8,749	18,991
Total current liabilities	$ 38,678	$ 40,056

Deferred revenue - net of current portion	9,632	—
Other long term liabilities	1,255	2,478
Total long term liabilities	10,887	2,478
Total liabilities	$ 49,565	$ 42,534
Stockholders' equity		
Common stock, par value of $0.0001 per share; 300,000,000 shares authorized, 54,723,455 and 54,032,826 shares issued and outstanding as of June 30, 2025 and December 31, 2024, respectively	5	5
Additional paid-in capital	736,276	719,997
Accumulated other comprehensive income (loss)	96	(151)
Accumulated deficit	(401,434)	(490,830)
Total stockholders' equity	$ 334,943	$ 229,021
Total liabilities and stockholders' equity	$ 384,508	$ 271,555

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

	Three Months Ended June 30,		Six Months Ended June 30,	
	2025	2024	2025	2024
Revenue	$ 13,817	$ 4,831	$ 172,386	$ 9,048
Operating expenses:				
Research and development	25,855	21,136	58,531	43,504
General and administrative	15,262	13,037	29,915	23,258
Total operating expenses	41,117	34,173	88,446	66,762
Income (loss) from operations	(27,300)	(29,342)	83,940	(57,714)
Other income (expense):				
Interest income (expense), net	3,789	3,695	6,678	6,121
Other income (expense), net	28	(48)	57	(476)
Total other income (expense)	3,817	3,647	6,735	5,645
Income (loss) before income taxes	$ (23,483)	$ (25,695)	$ 90,675	$ (52,069)
Provision for income taxes	—	—	1,278	—
Net income (loss)	$ (23,483)	$ (25,695)	$ 89,397	$ (52,069)
Net income (loss) per share:				
Basic	$ (0.40)	$ (0.46)	$ 1.54	$ (1.02)
Diluted	(0.40)	(0.46)	$ 1.50	(1.02)
Weighted-average common shares outstanding:				
Basic	58,353,855	55,765,948	$ 58,109,622	51,288,222
Diluted	58,353,855	55,765,948	$ 59,681,472	51,288,222
Comprehensive income (loss):				
Net income (loss)	$ (23,483)	$ (25,695)	$ 89,397	$ (52,069)
Other comprehensive gain (loss):				
Unrealized gain (loss) on marketable securities	200	(15)	247	9
Total other comprehensive gain (loss)	$ 200	$ (15)	$ 247	$ 9
Comprehensive income (loss)	$ (23,283)	$ (25,710)	$ 89,644	$ (52,060)

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