Stoke Therapeutics Appoints Thomas McCauley, Ph.D., as Chief Scientific Officer

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–Dr. McCauley brings more than 25 years of experience building high-performing R&D organizations and translating early scientific discoveries into new medicines for people with severe diseases–

BEDFORD, Mass.--(BUSINESS WIRE)--Jul. 13, 2026-- 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. Today, the Company announced that Thomas McCauley, Ph.D., has been appointed Chief Scientific Officer.

Dr. McCauley joins Stoke with more than 25 years of experience leading high-performing R&D teams and translating early scientific discoveries into clinical programs that have delivered new medicines to people living with severe diseases. He brings extensive expertise in developing novel technology platforms and modalities across multiple therapeutic areas, including six years at Shire where he played a key role in the late-stage development and global approvals of multiple genetic medicines for rare diseases. In this new role, he will be responsible for leading Stoke's scientific strategy and leveraging the Company's proprietary RNA medicines platform to expand and advance its pipeline.

"Our first investigational medicine, zorevunersen, has shown the potential to change the course of Dravet syndrome by addressing the underlying genetic cause of the disease," said Ian F. Smith, Chief Executive Officer and Director of Stoke Therapeutics. "As we build on this proof-of-concept for our platform and expand our pipeline beyond Dravet, Tom's leadership and deep translational expertise will help us identify and advance the best opportunities to turn novel science from our research organization into more life-changing medicines for patients."

"Throughout my career, I have had the opportunity to drive the development of medicines across a wide range of modalities and scientific platforms. Stoke's approach of upregulating protein expression stood out to me for its potential to transform the way many severe genetic diseases are treated," said Dr. McCauley. "I am very impressed and energized by the progress the team has already made, including advancing a potential first-in-class disease-modifying medicine for Dravet syndrome from early research into late-stage development. I look forward to partnering with Stoke's co-founder and Chief Technology Officer Isabel Aznarez and the broader organization to build on that foundation and help realize the full potential of this promising scientific approach."

Dr. McCauley most recently served as the President and Chief Executive Officer of Neptune Bio, where he drove the company's strategic vision and advanced therapeutic applications for its scientific platform. Dr. McCauley previously served as the Chief Scientific Officer at Omega Therapeutics, Macrolide Pharmaceuticals and Translate Bio, where he led the advancement of each company's scientific platform and drug development capabilities. Earlier in his career, Dr. McCauley held leadership and scientific roles at Shire, Inotek Pharmaceuticals, and Archemix Corp. In these roles, he cultivated expertise in areas such as rare diseases and mRNA technologies. He currently serves as a Scientific Advisor for multiple early-stage biotechnology companies in the RNA and genetic therapeutics space.

Dr. McCauley holds undergraduate and graduate degrees in Applied & Engineering Physics from Cornell University and received his Ph.D. in Physics from the University of Alabama at Birmingham.

About Dravet Syndrome

Dravet syndrome is a severe developmental and epileptic encephalopathy (DEE) characterized by 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. Even when treated with the best available anti-seizure medicines (ASMs), up to 57% of patients with Dravet syndrome do not achieve ≥50% reduction in seizure frequency. 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; up to 20% of children and adolescents with Dravet syndrome die before adulthood due to SUDEP, prolonged seizures, seizure-related accidents or infections[1]. 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[2]. There are no approved disease-modifying therapies for people living with Dravet syndrome.

About Zorevunersen

Zorevunersen is an investigational antisense oligonucleotide that is designed to treat the underlying cause of Dravet syndrome by increasing functional NaV1.1 protein production in brain cells from the unaffected (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, and China's Center for Drug Evaluation has

granted zorevunersen Breakthrough Therapy Designation. Stoke has a strategic collaboration with Biogen (Nasdaq: BIIB) 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. Zorevunersen is currently in clinical development, and its safety and efficacy have not been evaluated by any regulatory authority.

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/ and follow us on LinkedIn.

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 ability to expand and advance its pipeline using its proprietary RNA medicines platform; and the potential benefits, safety and efficacy of zorevunersen. Statements including words like "will," "potential," or similar words 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; the potential that 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 into 2028; and the other risks and uncertainties described under the heading "Risk Factors" in its Annual Report on Form 10-K for the year ended December 31, 2025, 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.

References:

1. Symonds, J. et al. Early childhood epilepsies: epidemiology, classification, aetiology, and socio-economic determinants. *Brain.* 2021;144(9):2879-2891.
2. 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.

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