Stoke Therapeutics Appoints Bo Cumbo to its Board of Directors, Enhancing Commercial Expertise as the Company Advances Zorevunersen Development Toward Potential FDA Approval and Commercialization

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–Mr. Cumbo brings deep experience commercializing new medicines for people with rare genetic diseases–

–Company announces retirement of Director Edward Kaye, M.D., from the Board–

BEDFORD, Mass.--(BUSINESS WIRE)--Sep. 23, 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 the appointment of Bo Cumbo to its Board of Directors. Concurrent with Mr. Cumbo's appointment, Edward Kaye, M.D., has retired from his role as a Director on Stoke's Board. Dr. Kaye has served on Stoke's Board of Directors since 2017 and was the Chief Executive Officer of the Company from 2017 to March 2025. Dr. Kaye will continue to advise Stoke's Board of Directors through the transition.

"Bo's track record of commercializing medicines for rare pediatric diseases will further enhance the capabilities of our Board as we prepare to deliver zorevunersen to people with Dravet syndrome following potential FDA approval in early 2028," said Ian F. Smith, Chief Executive Officer and Director of Stoke Therapeutics. "Bo also brings experience leading organizations through significant growth, which will help position Stoke for long-term success as we build our company for the future. We are very pleased to welcome Bo to the Board."

Mr. Smith continued, "On behalf of the Board, I would like to thank Ed Kaye for his many years of service and contributions to Stoke, including supporting the team and me over the past 18 months as I assumed the role of CEO."

Mr. Cumbo has more than 30 years of experience in the biopharmaceutical industry, spanning commercial leadership, new medicine launches and corporate governance. Since 2022 he has served as President, Chief Executive Officer and a Director of Solid Biosciences. He was previously the founding Chief Executive Officer of AavantiBio. Between 2013 and 2020, he held several commercial leadership positions of increasing responsibility at Sarepta Therapeutics, where he ultimately served as Chief Commercial Officer and was instrumental in bringing the first FDA-approved treatments for Duchenne muscular dystrophy to patients. Earlier in his career, he held leadership roles at Vertex Pharmaceuticals, Gilead Sciences, GlaxoSmithKline and UCB Pharma. Mr. Cumbo has extensive board experience, currently serving as a Director of both Vor Bio and Climb Bio and previously serving as a Director of Verve Therapeutics, RA Pharma and Clinical Supplies Management. He holds a Bachelor of Science in Laboratory Technology from Auburn University.

"Throughout my career, I have played a role in bringing first-of-their-kind, genetically targeted medicines to people living with rare, debilitating diseases, and witnessed the impact these treatments have on patients and their families," said Mr. Cumbo. "Stoke is developing the first potential medicine designed to address the underlying cause of Dravet syndrome with the goal of both reducing seizures and improving neurodevelopment for people living with this disease. Beyond Dravet syndrome, the Company's scientific approach holds the potential to address the root cause of many other severe genetic diseases. I am excited to join the Board at such an important stage and support the team as they advance zorevunersen and expand the pipeline."

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 percent of patients with Dravet syndrome do not achieve ≥50 percent 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 percent 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.

Stoke Therapeutics 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 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 timing and results of regulatory submissions or decisions for zorevunersen; and the potential for the Company's scientific approach to address the root cause of many other genetic diseases. Statements including words such as "plan," "anticipate", "potential," "will," "continue," "may", "expect," 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; that if collaborators were to breach or terminate their agreements, the Company would not obtain the anticipated financial or other benefits; 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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