Stoke Therapeutics Announces First Patient Dosed in Phase 1 Study of STK-002, a Potential Disease-Modifying Medicine for the Treatment of Autosomal Dominant Optic Atrophy (ADOA)

February 11, 2026

–Dose-escalating study will evaluate the safety, tolerability and exposure of STK-002 in people with ADOA–
–Changes in visual function, ocular structure and quality of life to be evaluated as secondary objectives–

–ADOA is the most common inherited optic nerve disorder and is primarily caused by variants in the OPA1 gene that result in progressive and irreversible vision loss–

BEDFORD, Mass.--(BUSINESS WIRE)--Feb. 11, 2026-- Stoke Therapeutics, Inc. (Nasdaq: STOK), a biotechnology company dedicated to restoring protein expression by harnessing the body's potential with RNA medicine, today announced that the first patient has been dosed in the Phase 1 OSPREY study of STK-002, an investigational medicine for the treatment of Autosomal Dominant Optic Atrophy (ADOA). ADOA is a rare genetic disease that causes progressive and irreversible vision loss in both eyes starting in the first decade of life. Approximately 80 percent of people with ADOA are symptomatic by age 10, and approximately half are expected to become legally blind. There are currently no approved treatments for ADOA.

"Data from our natural history study suggest that for some people affected by ADOA, the disease progresses more rapidly than previously thought. Based on a growing understanding of the disease biology, we believe that increasing naturally occurring OPA1 protein may help restore vision in people with ADOA," said the lead principal investigator of the study Dr. Patrick Yu-Wai-Man, M.D., Ph.D., Professor of Ophthalmology at the University of Cambridge and honorary consultant neuro-ophthalmologist at Addenbrooke's Hospital, Moorfields Eye Hospital, and the UCL Institute of Ophthalmology, United Kingdom. "There are currently no medicines available for people living with ADOA, and there is a lot of interest in this study from the ADOA community given the potential for STK-002 to restore vision by addressing the root cause of the disease."

The OSPREY study is a Phase 1, dose-escalating open-label study of children and adults ages 6 to 55 who have an established diagnosis of ADOA and have a confirmed disease-causing variant in the *OPA1* gene. The primary objectives for the study are to assess the safety and tolerability of single ascending doses of STK-002, as well as to determine the exposure in blood. Secondary objectives are to assess changes in visual function, ocular structure and quality of life after single doses of STK-002. The OSPREY study follows a standard dose escalation design with participants enrolled into sequential cohorts receiving increasing dose levels of STK-002. Dose escalation of the first four cohorts will continue through 2026 and early 2027, pending safety and tolerability assessments. Data from the OSPREY study will help to inform potential future development of STK-002.

"ADOA is a haploinsufficient disease, one of many that we believe are ideally suited for our ASOs that are designed to increase naturally occurring protein levels to improve health," said Barry Ticho, M.D., Ph.D., Chief Medical Officer of Stoke Therapeutics. "We are pleased to be expanding our approach into a new disease area, leveraging our learnings from Dravet syndrome and applying them to the development of a potential disease-modifying medicine for people living with ADOA."

The OSPREY study is actively recruiting in the United Kingdom and Germany. Additional European sites are expected to activate in the coming months.

About Autosomal Dominant Optic Atrophy (ADOA)

ADOA is the most common inherited optic nerve disorder, affecting approximately one in 30,000 people globally with a higher incidence of one in 10,000 in Denmark due to a founder effect. 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. Approximately half of people with ADOA fail driving standards and up to 46% are registered as legally blind. More than 400 different disease-causing *OPA1* variants have been reported in people diagnosed with ADOA. Currently there are no approved treatments for people living with ADOA.

About STK-002

STK-002 is a proprietary antisense oligonucleotide (ASO) in clinical development for the treatment of ADOA. 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 ADOA cases are caused by variants 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 people 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 U.S. Food and Drug Administration (FDA) as a potential new treatment for ADOA. A Phase 1 study (OSPREY) of STK-002 in people with ADOA is now underway.

About the Phase 1 OSPREY Study

The OSPREY study is a Phase 1, dose-escalating open-label study of children and adults ages 6 to 55 who have an established diagnosis of ADOA and have a confirmed disease-causing variant in the *OPA1* gene. The primary objectives for the study are to assess the safety and tolerability of single ascending doses of STK-002, as well as to determine the exposure in blood. Secondary objectives are to assess changes in visual function, ocular structure and quality of life after single doses of STK-002. The OSPREY study follows a standard dose escalation design with participants enrolled into sequential cohorts receiving increasing dose levels of STK-002. Dose escalation of the first four cohorts will continue through 2026 and early 2027, pending safety and tolerability assessments. Data from the OSPREY study will help to inform potential future development of STK-002. The OSPREY

study is actively recruiting in the United Kingdom and Germany. Additional European sites are expected to activate in the coming months.

For more information on the OSPREY study, please visit:

- https://www.ospreyclinicaltrial.com/
- https://www.isrctn.com/ISRCTN41725621

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 ability of STK-002 to treat the underlying cause of ADOA and maintain or improve vision; and the timing, details and expected progress of clinical trials for STK-002. Statements including words such as "anticipate," "potential", "could," "expect," "plan," "will," "may" 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 Stoke'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: Stoke's ability to advance, obtain regulatory approval and ultimately commercialize its product candidates; that if Stoke's collaborators were to breach or terminate their agreements, it would not obtain the anticipated financial or other benefits; the possibility that Stoke and its collaborators may not be successful in their development of product candidates and that, even if successful, they may be unable to successfully commercialize such product candidates; 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; Stoke's ability to protect its intellectual property; Stoke'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 Stoke'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 Stoke undertakes no obligation to revise or update any forward-looking statements to reflect events or circumstances after the date hereof.

View source version on businesswire.com: https://www.businesswire.com/news/home/20260211690284/en/

Media & Investor Contacts:
Susan Willson
Vice President, Corporate Communications
swillson@stoketherapeutics.com
415-509-8202

Doug Snow
Director, Communications & Investor Relations
IR@stoketherapeutics.com
508-642-6485

Source: Stoke Therapeutics, Inc.