



## Stoke Therapeutics Announces Presentations of STK-002, an Investigational Medicine for the Treatment of Autosomal Dominant Optic Atrophy (ADOA), at the American Academy of Ophthalmology (AAO) 2026 Annual Meeting

October 6, 2026

*–ADOA is the most common inherited optic nerve disorder and is primarily caused by variants in the OPA1 gene that result in insufficient levels of OPA1 protein, leading to progressive and irreversible vision loss–*

*–Preclinical data and progress of the Phase 1 OSPREY study of STK-002 in people with ADOA will be presented at AAO 2026–*

*–Dosing is now complete in first two cohorts of OSPREY study, with dosing of third cohort expected to complete by year-end 2026; initial safety and efficacy data anticipated in H1 2027–*

BEDFORD, Mass.--(BUSINESS WIRE)--Oct. 6, 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 two presentations of data for STK-002 at the American Academy of Ophthalmology (AAO) 2026 Annual Meeting, taking place October 9-12 in New Orleans. STK-002 is an investigational medicine designed to target the underlying cause of Autosomal Dominant Optic Atrophy (ADOA) by increasing OPA1 protein levels to improve vision in people living with the disease. ADOA is the most common inherited optic nerve disorder. There are currently no approved treatments for ADOA. Dose escalation is continuing in the Phase 1 OSPREY study of STK-002 in people with ADOA, with the first two dose cohorts now complete and the third cohort expected to complete by year-end 2026. Cohorts three and four are expected to achieve therapeutic levels of dosing, with initial safety and efficacy data from these latter cohorts anticipated in the first half of 2027.

"ADOA causes progressive and irreversible vision loss in both eyes with 80% of patients symptomatic by age 10 and approximately half progressing to legal blindness," said Barry Ticho, M.D., Ph.D., Chief Medical Officer of Stoke Therapeutics. "We are encouraged by our preclinical data as well as emerging findings from the field demonstrating that upregulation of OPA1 protein may have disease-modifying potential. We continue to advance the Phase 1 OSPREY study of STK-002 in people with ADOA, with dose escalation progressing into higher cohorts expected to reach therapeutic levels. We anticipate initial results in the first half of next year to guide our next steps for development."

Data to be presented at AAO 2026 provide translational evidence that increasing functional OPA1 protein may have disease-modifying potential in ADOA. STK-002 is designed to increase the amount of functional mRNA produced by the healthy copy of the *OPA1* gene, resulting in more functional OPA1 protein. STK-002 represents a novel approach to ADOA designed to target the underlying disease biology.

Details of the presentations are as follows:

- **Title:** [OSPREY: First-in-Human Phase 1 Study of the RNA-Based Therapy STK-002 in Autosomal Dominant Optic Atrophy](#)  
**Podium Poster Date & Time:** Sunday, October 11, 10:30-10:40 AM CT  
**Presenting Author:** Patrick Yu-Wai-Man, M.D., Ph.D., Professor of Ophthalmology at the University of Cambridge and the UCL Institute of Ophthalmology, and Honorary Consultant Neuro-ophthalmologist at Addenbrooke's Hospital and Moorfields Eye Hospital, United Kingdom  
**Poster Number:** PO055
- **Title:** [RNA-Based OPA1 Upregulation in Autosomal Dominant Optic Atrophy: First-in-Human STK-002 Study in Mitochondrial Optic Neuropathy](#)  
**Poster Panel Discussion Date & Time:** Sunday, October 11, 3:15–3:21 PM CT  
**Presenting Author:** Nancy J. Newman, M.D., Professor, Departments of Ophthalmology, Neurology and Neurological Surgery, Emory University School of Medicine  
**Poster Number:** PO222

### About Autosomal Dominant Optic Atrophy (ADOA)

ADOA is the most common inherited optic nerve disorder, affecting approximately one in 25,000 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. 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, of which more than 600 have been reported in people diagnosed with ADOA. Most of these variants in the *OPA1* gene 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 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 FDA as a potential new treatment for ADOA. A Phase 1 study (OSPREY) of STK-002 in people with ADOA is ongoing.

#### 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. Dosing of the first two cohorts of patients is complete, with dosing of the third cohort expected to complete by year-end 2026. Subject to ongoing safety assessments, dosing of the fourth cohort is expected to follow. Initial safety and efficacy results are anticipated in the first half of 2027. Data from the OSPREY study will help to inform potential future development of STK-002.

All eight planned OSPREY clinical trial sites are now active in the UK, Germany, Denmark, Italy and Austria. 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/> or 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 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; and the participation of scientists associated with the Company making presentations at AAO and the presentation of data at AAO. 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 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 the Company's collaborators were to breach or terminate their agreements, the Company would not obtain the anticipated financial or other benefits; the possibility that the Company 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; 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.

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