



Stoke Therapeutics Presents Data Supporting the Disease-Modifying Potential of STK-002 in Autosomal Dominant Optic Atrophy (ADOA) at the American Academy of Ophthalmology (AAO) 2026 Annual Meeting

October 12, 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–

–STK-002 represents a novel approach to ADOA by targeting the underlying disease biology, with the potential to be the first disease-modifying medicine for people living with ADOA–

–ADOA natural history findings and preclinical proof-of-concept data for STK-002 support the ongoing Phase 1 OSPREY study in people with ADOA–

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

BEDFORD, Mass.--(BUSINESS WIRE)--Oct. 12, 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 represents a novel approach to Autosomal Dominant Optic Atrophy (ADOA), the most common inherited optic nerve disorder, by targeting the underlying disease biology. ADOA natural history findings and preclinical data for STK-002 support the ongoing Phase 1 OSPREY study and the disease-modifying potential of STK-002 in ADOA.

Dose escalation is ongoing in the OSPREY study, with the first two dose cohorts now complete and the third cohort expected to complete by the end of 2026. Cohorts three and four have the potential to reach therapeutic levels of dosing, with initial safety and efficacy data from these latter cohorts anticipated in the first half of 2027.

"ADOA can cause severe progressive visual impairment often beginning in childhood, and there are currently no approved treatments," said Patrick Yu-Wai-Man, M.D., Ph.D., Professor of Ophthalmology at the University of Cambridge and the UCL Institute of Ophthalmology, Honorary Consultant Neuro-ophthalmologist at Addenbrooke's Hospital and Moorfields Eye Hospital, United Kingdom, and the lead investigator on the Phase 1 OSPREY study. "These early data are an important step in evaluating whether an RNA-based medicine can address the underlying OPA1 deficiency and ultimately improve visual outcomes for people with ADOA."

"We're encouraged by the progress in the Phase 1 OSPREY study of STK-002 in people with ADOA, with the first two dose cohorts now complete," said Barry Ticho, M.D., Ph.D., Chief Medical Officer of Stoke Therapeutics. "Dose cohorts three and four are designed to show measurable effects on visual function, and we look forward to safety and efficacy results from these latter cohorts in the first half of next year."

The data presented at AAO 2026 span STK-002's preclinical and early clinical development, linking insights from ADOA disease biology and the FALCON natural history study to preclinical findings and the ongoing Phase 1 OSPREY study of STK-002 in people with ADOA.

Data Presented at the AAO 2026 Annual Meeting Include:

- **ADOA Disease Biology:** ADOA is the most common inherited optic nerve disorder primarily caused by variants in the *OPA1* gene, which lead to a haploinsufficiency resulting in 50% *OPA1* protein expression. Functional *OPA1* protein supports mitochondrial functioning, which is critical to meet the high energy demands of the retinal ganglion cells that make up the optic nerve. Insufficient *OPA1* protein leads to mitochondrial dysfunction, degeneration of the optic nerve, and ultimately vision loss.
- **ADOA Natural History:** Results from the FALCON natural history study show patients with ADOA experience higher levels of mitochondrial oxidative stress compared with healthy individuals, which strongly correlate with decreased visual acuity. These data support the mechanistic rationale that upregulation of the *OPA1* protein may have disease-modifying potential in ADOA.
- **Preclinical Proof-of-Concept:** 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. Across preclinical studies, treatment with STK-002 has demonstrated dose-dependent increases in retinal exposure, target engagement and *OPA1* protein expression, as well as improved mitochondrial function in ADOA patient-derived cells with different *OPA1* variants.
- **Clinical Progress with OSPREY:** OSPREY is a Phase 1, first-in-human dose-escalation study evaluating the safety and tolerability of STK-002 in people with *OPA1*-associated ADOA. OSPREY will also evaluate whether STK-002's ability to

upregulate OPA1 expression translates into measurable effects on visual function. 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 in the fourth cohort is expected to follow. Initial safety and efficacy results are anticipated in the first half of 2027.

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 the end of 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 the FALCON Study

FALCON was a multicenter, 24-month, prospective natural history study of people ages 8 to 60 who are living with ADOA. FALCON aimed to provide a better understanding of how ADOA disease parameters change over time to inform potential future interventional clinical trials and was designed to evaluate the rate of change in structural and functional ophthalmic assessments. No investigational medications or other treatments were provided. The study enrolled 47 patients across 10 sites in the United States, United Kingdom, Italy and Denmark. All participants had a confirmed diagnosis of ADOA caused by an *OPA1* variant. Patients underwent assessments at baseline, 6 months, 12 months, 18 months and 24 months. Data from the FALCON study support the clinical development of STK-002, Stoke's proprietary antisense oligonucleotide (ASO) currently being evaluated in the Phase 1 OSPREY study.

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; the timing, details and expected progress of clinical trials and data readouts for STK-002; and the potential for dose cohorts three or four in the OSPREY study to achieve therapeutic levels of dosing. Statements including words such as "anticipate," "expect," "potential," "could," "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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Source: Stoke Therapeutics, Inc.