Atsena Therapeutics Doses First Patient and Reports Rapid Early Enrollment in Pivotal Trial of ATSN-201 for X-Linked Retinoschisis

Enrollment expected to complete Q1 2027; topline results anticipated 1H 2028; BLA filing targeted for 2H 2028

Phase 1/2 data demonstrated ATSN-201's ability to reverse structural damage to the retina and improve vision in patients with XLRS

DURHAM, NC, June 22, 2026 — Atsena Therapeutics, a clinical-stage gene therapy company focused on using the life-changing power of genetic medicine to reverse or prevent blindness, today announced that the first patient has been dosed in the Phase 3 pivotal cohort of the LIGHTHOUSE trial evaluating ATSN-201 for the treatment of X-linked retinoschisis (XLRS).

Since beginning enrollment in May, Atsena has already enrolled 10% of the study and expects rapid continued enrollment as additional planned sites are activated across North America and Europe. Atsena expects to complete enrollment by the end of the first quarter of 2027, with topline results anticipated in the first half of 2028 and a Biologics License Application (BLA) filing targeted for the second half of 2028.

“Brisk enrollment of the study reflects the significant unmet need in XLRS, a disease for which no approved treatments exist, and strong interest from leading clinicians and patients who have been following ATSN-201's clinical progress,” said Kenji Fujita, MD, Chief Medical Officer of Atsena. “That interest is grounded in compelling early clinical results. The majority of treated patients in the Phase 1/2 portion of the LIGHTHOUSE trial demonstrated closure of schisis cavities and meaningful improvements in retinal and visual function at levels consistent with FDA approvability, outcomes that have proven durable through at least one year of follow-up.”

"Dosing the first patient in the LIGHTHOUSE pivotal cohort is a defining moment for Atsena and for the XLRS community," said Patrick Ritschel, Chief Executive Officer of Atsena. "The speed with which we are enrolling speaks to the urgency patients and physicians have about finding a treatment for this disease. We enter this pivotal study with a high degree of confidence, backed by Phase 1/2 data showing that ATSN-201 can reverse structural damage to the retina and meaningfully improve vision. XLRS families have waited a long time for a treatment, and we intend to deliver one."

About the LIGHTHOUSE Phase 3 Pivotal Cohort

The Phase 3 pivotal portion of the LIGHTHOUSE trial is enrolling 76 patients with XLRS, including both adults and children as young as age 6, across leading medical centers in North America and Europe. Patients are randomized approximately 1:1 between a treatment arm, which receives ATSN-201, and a control arm, which is observed for 12 months and then offered the option to receive treatment. The primary endpoint is change in microperimetry at 52 weeks, as aligned with both the U.S. Food and Drug Administration (FDA) and the European Medicines Agency (EMA). Key secondary endpoints include visual acuity and optical coherence tomography (OCT)-based measures of retinal structure.

About X-linked Retinoschisis (XLRS)

XLRS is a monogenic X-linked disease caused by mutations in the *RS1* gene which encodes retinoschisin, a protein secreted primarily by photoreceptors. RS1 is localized to the extracellular surface of rods,

cones and bipolar cells. XLRS is characterized by schisis, or abnormal splitting of retinal layers, which causes impaired visual acuity that is not correctable with glasses and leads to progressive vision loss and ultimately blindness. XLRS primarily affects males and is typically diagnosed in early childhood. Approximately 30,000 males in the U.S. and EU have XLRS, for which there are currently no approved treatments.

About ATSN-201
ATSN-201 is Atsena's investigational gene therapy leveraging AAV.SPR, a novel, laterally spreading capsid designed to efficiently target photoreceptors in the central retina while avoiding the surgical risks of foveal detachment. It is currently being evaluated in the Phase 1/2/3 LIGHTHOUSE Trial. The Phase 3 pivotal cohort is actively enrolling.

ATSN-201 is the first XLRS gene therapy to demonstrate preliminary evidence of efficacy and safety in a Phase 1/2 trial, with the majority of patients demonstrating improvements in retinal structure (foveal schisis closure) and meaningful improvements in retinal and visual function as assessed by microperimetry, best-corrected visual acuity and low-luminance visual acuity. ATSN-201 has demonstrated a favorable safety profile and has been well-tolerated for at least one year post-treatment. This best-in-class gene therapy product candidate has received Regenerative Medicine Advanced Therapy, Fast Track, Rare Pediatric Disease and Orphan Drug Designations from the U.S. Food and Drug Administration and Orphan Designation from the European Medicines Agency. For more information, visit ClinicalTrials.gov (Identifier: NCT05878860).

About AAV.SPR
AAV.SPR, one of Atsena's novel capsids, spreads laterally beyond the subretinal injection site to enable safe and efficient transduction of the central retina when injected into areas outside the macula. A preclinical study in non-human primates demonstrated that AAV.SPR promotes transgene expression well beyond subretinal injection bleb margins. This is in contrast to benchmark AAV vectors, which remain confined to the original bleb margins. At clinically relevant doses, AAV.SPR efficiently transduces foveal cones without the need for surgical detachment and has a favorable safety profile relative to benchmark capsids. For more information about the preclinical study and how AAV.SPR works, visit https://atsenatx.com/our-approach/laterally-spreading-aav/.

About Atsena Therapeutics
Atsena is a clinical-stage gene therapy company developing best-in-class treatments for the reversal or prevention of blindness from inherited retinal diseases. The company's lead program is evaluating ATSN-201 in a pivotal clinical trial for X-linked retinoschisis (XLRS), a genetic condition that is typically diagnosed in childhood and leads to blindness later in life. The company's proprietary pipeline also includes gene therapies in development for Usher Syndrome Type 1B and for Stargardt Disease. Atsena is also developing ATSN-101, a first-in-class, investigational gene therapy for Leber congenital amaurosis type 1 (LCA1), as part of its exclusive strategic collaboration with Nippon Shinyaku Co., Ltd. ATSN-101 has completed a Phase 1/2 trial with positive results (https://doi.org/10.1016/s0140-6736(24)01447-8), and Atsena expects to initiate a global pivotal Phase 3 clinical trial evaluating ATSN-101 in the second half of 2026.

Founded by pioneers in ocular gene therapy, Atsena is led by an experienced team dedicated to addressing the needs of patients with vision loss. For more information, please visit https://atsenatx.com/.

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