Ascidian Completes Dose Escalation Portion of Phase 1/2 STELLAR Trial of ACDN-01 for Stargardt Disease and Announces Expanded Clinical Study Plans

STELLAR to begin enrolling pediatric subjects

STARPATH prescreening study open for adults and children with Stargardt disease to streamline entry into future ACDN-01 clinical trials

BOSTON, May 4, 2026 – Ascidian Therapeutics, a biotechnology company seeking to treat human diseases by rewriting RNA, today announced the completion of the adult dose escalation portion of the STELLAR Phase 1/2 clinical trial of ACDN-01 in Stargardt disease, and expansion of the STELLAR study to pediatric subjects over 12 years of age.

The first-in-human safety data on ACDN-01 from the STELLAR study will be presented at the American Society of Gene & Cell Therapy Annual Meeting on May 12th.

STELLAR is a multi-center, open-label, dose-escalation study evaluating ACDN-01, Ascidian's lead RNA exon editor candidate. The trial assesses the safety and preliminary efficacy of a single subretinal injection in people with Stargardt disease and other *ABCA4* retinopathies. The dose escalation portion of the study enrolled 10 adult participants (ages 18-77) and is now enrolling pediatric subjects ages 12 and older.

To support future clinical development in Stargardt disease, Ascidian has also initiated STARPATH, an observational study for adults and children ages 5 and older. Participants receive genetic testing and high-resolution retinal imaging to better characterize their disease and assess eligibility for future ACDN-01 trials.

"ACDN-01 offers a novel, differentiated approach to correcting the genetic basis of Stargardt disease," said **Michael Ehlers, M.D., Ph.D.**, Founder, President, and Chief Executive Officer of Ascidian Therapeutics. "By targeting RNA, ACDN-01 has the potential to address the root cause of Stargardt disease, using a single AAV vector, without altering DNA or introducing foreign enzymes. Ascidian's goal is to turn pioneering science into life-changing solutions for families living with this blinding condition and today is an important step towards that goal."

"The Stargardt community has long awaited a therapy that addresses the genetic complexity of this disease," said **Mark Pennesi, M.D., Ph.D., FARVO**, Chief Medical Officer at the Retina Foundation of Dallas and Adjunct Professor of Ophthalmology at the Oregon Health & Science University Casey Eye Institute. "With adult dose escalation complete and pediatric subjects now enrolling, we have the opportunity to evaluate ACDN-01 across a broader range of subjects, including those earlier in their disease, where intervention may have the greatest impact."

ACDN-01, the only clinical-stage, single vector product in development which targets the underlying cause of Stargardt disease, performs *in vivo* RNA exon editing and restores full-length

ABCA4 protein that is deficient in Stargardt disease – without modifying the genome or introducing foreign enzymes. This first-in-class approach has already demonstrated durable and efficient *ABCA4* exon editing in both non-human primate and human retinal explant models.

To learn more about STELLAR and STARPATH, visit AscidianClinicalTrials.com.

About Stargardt Disease

Stargardt disease is the most common form of inherited macular degeneration and currently has no FDA-approved treatments. Affecting approximately 30,000 individuals in the U.S. alone, Stargardt disease is caused by mutations in the *ABCA4* gene which lead to progressive retinal degeneration and vision loss, typically beginning in childhood and young adulthood. More than 1,000 mutations across the *ABCA4* gene have been found to cause Stargardt disease. Diseases caused by *ABCA4* loss of function – including Stargardt disease – are examples of genetic disorders that cannot be addressed by standard gene replacement, given the large size of the gene, or by base editing, due to the high mutational variance of the affected gene.

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About Ascidian Therapeutics

Ascidian Therapeutics is redefining the treatment of disease by rewriting RNA. By editing exons at the RNA level, Ascidian therapies enable precise post-transcriptional editing of genes, resulting in full-length, functional proteins at the right levels, in the right cells, at the right time. With partnered and wholly owned programs in retinal, neurological, neuromuscular, and genetically defined diseases, Ascidian's approach has the potential to treat patients with one dose of an RNA exon editor, opening new therapeutic possibilities for patients and their families who are seeking breakthroughs.

Learn more at www.ascidian.com, or for more information about STELLAR and STARPATH, visit www.AscidianClinicalTrials.com.

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