Ascidian Therapeutics to Present Preclinical Data for RNA Exon Editor, ACDN-01, Supporting an Open IND for Phase 1/2 Clinical Testing at ASGCT 2024 Annual Meeting

Compelling translational insights underscore therapeutic potential of Ascidian's lead ACDN-01 program for people living with Stargardt disease and other ABCA4 *retinopathies*

ACDN-01 awarded Rare Pediatric Disease Designation, following U.S. FDA IND clearance and Fast Track Designation announced earlier this year

BOSTON, April 25, 2024 – Ascidian Therapeutics, a biotechnology company seeking to treat human diseases by rewriting RNA, today announced that it will share preclinical data that enabled IND clearance for Phase 1/2 testing for its lead RNA exon editing therapy, ACDN-01, in a poster session at the American Society of Gene & Cell Therapy (ASGCT) annual meeting in Baltimore, MD, May 7-11, 2024.

The company also announced that it was granted Rare Pediatric Disease Designation for ACDN-01 from the U.S. Food and Drug Administration (FDA). This action by the FDA follows Fast Track Designation and IND clearance announced earlier this year. ACDN-01 is the first-ever RNA exon editor to enter clinical development and the first RNA editing therapeutic to be cleared for clinical investigation in the United States prior to any other geography.

"ACDN-01 is the only clinical-stage therapeutic targeting the genetic cause of Stargardt disease, and we look forward to sharing the preclinical data that led to its IND clearance, along with the plans for the first-in-human trial," said **Michael Ehlers, M.D., Ph.D**., President and Interim Chief Executive Officer of Ascidian Therapeutics. "ACDN-01 is designed to overcome challenges that have long kept Stargardt disease out of reach and to provide persistent therapeutic benefit with one dose. Our progress with ACDN-01 speaks to its therapeutic potential in Stargardt disease and the broader promise of our RNA exon editing platform to significantly expand the possibilities of RNA medicines."

Ascidian Translational Science Research Fellow **Ting-Wen Cheng, Ph.D.**, will present the ACDN-01 preclinical dataset, including human retinal explant and primate GLP safety studies, that enabled the STELLAR clinical trial design. STELLAR is an open-label Phase 1/2 study evaluating the safety and efficacy of a single dose of ACDN-01, administered via subretinal injection in individuals with Stargardt disease and other *ABCA4* retinopathies.

Presentation details are below:

Session: Epigenetic Editing and RNA Editing
Abstract Number: 706
Poster Presentation: Exon Editing of *ABCA4* RNA in Human Retinal Explants and Non-Human Primate Retina for the Treatment of Stargardt Disease
Date: Wednesday, May 8, 2024
Time: 12:00 p.m. ET
Location: Exhibit Hall, Baltimore Convention Center

Presentation materials will be available upon request via the Publications & Presentations section of the Ascidian website following the live presentation.

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About Ascidian Therapeutics

Ascidian Therapeutics, an ATP company, 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 discovery, preclinical, and clinical 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. For more information, visit www.ascidian-tx.com.

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