Ascidian Therapeutics Announces First-Ever IND for an RNA Exon Editor as FDA Approves Trial Plan and Fast Tracks ACDN-01 in Stargardt Disease and Other *ABCA4* Retinopathies

ACDN-01 is the first RNA exon editor to enter clinical development and the only clinical-stage therapeutic targeting the genetic cause of Stargardt disease

Ascidian expects to initiate enrollment in Phase 1/2 STELLAR study in the first half of 2024

ACDN-01 also granted Fast Track designation by FDA

BOSTON, January 29, 2024 – Ascidian Therapeutics, a biotechnology company aspiring to treat human diseases by rewriting RNA, today announced that the U.S. Food and Drug Administration (FDA) has cleared its investigational new drug (IND) application and granted Fast Track designation for ACDN-01. ACDN-01 is the first-ever clinical-stage RNA exon editor and the only clinical-stage therapeutic targeting the genetic cause of Stargardt disease. Ascidian expects to initiate enrollment in the Phase 1/2 STELLAR study of ACDN-01 in Stargardt disease and other *ABCA4* retinopathies in the first half of 2024.

"This open IND for ACDN-01 by the FDA – the first regulator to have cleared ACDN-01 for clinical development – represents an important milestone for Ascidian and the broader field of RNA editing," said **Michael Ehlers, M.D., Ph.D.**, President and Interim Chief Executive Officer of Ascidian Therapeutics. "We chose to go to the FDA first because we have conviction in the rigor of our data, and that by editing RNA and not DNA, the Ascidian approach brings unique advantages with potential to transform the lives of people living with Stargardt disease and, more broadly, to dramatically expand the reach of genetic medicine."

"The advancement of Ascidian's first-of-its-kind RNA exon editor from the lab to the clinic is a unique and novel therapeutic approach targeting the genetic cause of Stargardt disease," said **Byron L. Lam, M.D.**, Director of the Mark J. Daily Inherited Retinal Disease Research Center at the Bascom Palmer Eye Institute, University of Miami Miller School of Medicine. "This is a critical step toward overcoming the challenges of Stargardt disease, such as the size of the *ABCA4* gene and large number of mutations within the patient population, that have long kept Stargardt out of reach for conventional gene therapies. Stargardt patients deserve treatment options, and I am looking forward to the clinical evaluation of this promising approach."

The open-label Phase 1/2 STELLAR study will evaluate the safety and efficacy of a single dose of ACDN-01, administered via subretinal injection in individuals with Stargardt disease and other *ABCA4* retinopathies.

Ascidian also announced that the FDA has granted Fast Track designation for ACDN-01. Fast Track designation is designed to facilitate the review of new medicines for serious conditions with high unmet need. This designation enables Ascidian to expedite development of ACDN-01 with regular feedback from FDA through the clinical-development process.

About Stargardt Disease and ACDN-01

Stargardt disease is the most common form of inherited macular degeneration and 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.

ACDN-01 is an *in vivo* RNA exon editor delivered by a single vector. It has demonstrated efficient, durable *in vivo* RNA exon editing in non-human primate retina and *ex vivo* RNA exon editing in human retinal explants.

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.com.

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