



Ascidian Therapeutics Raises \$40 Million in Series A Extension Financing from Apple Tree Partners and Appoints Michael Ehlers as Interim CEO

Funds from ATP will support the progress of Ascidian’s lead program in ABCA4 retinopathies, including Stargardt disease, and advance key pipeline programs in CNS and neuromuscular diseases

Ascidian anticipates filing an IND and initiating a first-in-patient trial in ABCA4 retinopathy in the first half of 2024

Ascidian Board of Directors reinstates Ascidian founding CEO Michael Ehlers to lead the company through its next period of growth

BOSTON, November 8, 2023 – [Ascidian Therapeutics](#), a biotechnology company focused on treating human diseases by rewriting RNA, today announced \$40 million in Series A extension funding committed by [Apple Tree Partners \(ATP\)](#). The funds will be used to advance Ascidian’s lead program, an RNA exon editor to halt the progression of Stargardt disease or other *ABCA4* retinopathies, and additional programs in the company’s pipeline. The company also announced that its founding CEO **Michael Ehlers, M.D., Ph.D.**, will return as interim President and Chief Executive Officer, effective immediately. Dr. Ehlers, who also chairs Ascidian’s Board of Directors and serves as Chief Scientific Officer at ATP as well as a venture partner at the firm, succeeds Romesh Subramanian, Ph.D., who is departing Ascidian.

“We thank Romesh for his contributions to Ascidian over the past 14 months, including his work to validate the company’s novel exon editing platform and progress its programs,” Dr. Ehlers said. “Ascidian’s approach to edit RNA at kilobase scales has demonstrated its potential in non-human primate studies, and today we are poised to submit our plans for human trials of what we believe will be a one-time treatment to preserve sight in people with Stargardt disease. This is only the beginning. The potential reach of altering RNA at this scale is vast.”

Today’s announced financing follows ATP’s initial \$50 million Series A investment in Ascidian announced in October 2022.

“ATP created and incubated Ascidian with Mike Ehlers as the framer of the vision and architect of its groundbreaking exon editing platform, which defines a new class of RNA therapeutics,” said **Seth Harrison, M.D.**, ATP founder and managing partner and a member of the Ascidian Board of Directors. “Our renewed investment in Ascidian is a resounding vote of confidence in the originality and brilliance of the company’s approach, the promising data to date, and Mike’s proven leadership.”

Ascidian designs RNA editors to replace multiple mutated exons simultaneously. Using this platform, Ascidian can edit genes too large to package in viral vectors and genes with high mutational variance, thereby addressing underlying causes of complicated genetic diseases that are beyond the reach of current gene therapy and base editing approaches. Because Ascidian’s technique does not introduce

exogenous enzymes, the risk of adverse immune reactions is reduced. And because it does not modify DNA, the risk of off-target effects is decreased.

Earlier this year, Ascidian presented at the American Society of Gene and Cell Therapy Annual Meeting (ASGCT) six-month data from its lead program establishing the production of therapeutically relevant levels of full-length ABCA4 protein following a one-time treatment with a single AAV-delivered RNA exon editing development candidate in the non-human primate retina. These data represent the most efficient and durable RNA exon editing via trans-splicing ever achieved in large animals.

Ascidian anticipates submitting an Investigational New Drug (IND) Application to the U.S. Food and Drug Administration (FDA) for its lead program in *ABCA4* retinopathies in early 2024. Beyond its lead program, Ascidian is advancing programs with first-in-class potential in neurological and neuromuscular disorders.

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 active discovery and preclinical 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 in need of breakthroughs. For more information, visit www.ascidian.com.

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