Ascidian Therapeutics to Present Data From Its Lead Program Targeting *ABCA4* Retinopathies at the Retinal Cell and Gene Therapy Innovation Summit and ARVO 2023 Annual Meeting

New six-month durability data from lead ABCA4 *program demonstrate the most efficient and durable RNA exon editing ever reported in a large animal*

Additional new data in human retinal explants is first-ever demonstration of RNA exon editing in human photoreceptors

BOSTON, April 18, 2023 – Ascidian Therapeutics, a biotechnology company focused on treating human diseases by rewriting RNA, announced today that Chief Medical Officer **Jay Barth, M.D.**, will present at the Eighth Annual Retinal Cell and Gene Therapy Innovation Summit on April 21, 2023, and Head of Molecular Biology **Shimyn Slomovic, Ph.D.**, will present at the Association for Research in Vision and Ophthalmology (ARVO) 2023 Annual Meeting on April 26, 2023. Their sessions will highlight Ascidian's groundbreaking RNA exon editing platform and the company's lead program targeting *ABCA4* retinopathies, including Stargardt disease, which is currently in IND-enabling studies.

Ascidian's first-of-its-kind RNA exon editing platform is designed to expand the therapeutic possibilities of genetic medicine and treat diseases not addressable by today's gene therapy and gene editing technologies. Data to be shared at ARVO demonstrate *ABCA4* RNA exon editing in multiple model systems, including cell lines, human retinal explants, and non-human primates at time points extending to six months.

"We look forward to sharing promising data from our lead program at the Retinal Cell and Gene Therapy Innovation Summit, ARVO, and other forthcoming medical meetings as we prepare to submit our IND," said **Romesh Subramanian, Ph.D.**, President and Chief Executive Officer of Ascidian Therapeutics. "At the same time, we are progressing our diversified pipeline of programs using our proprietary platform for rewriting RNA by exon editing in other retinal diseases, as well as neurological, neuromuscular, and other genetically defined diseases. Ascidian is leading the development of new ways to edit RNA for the treatment of human disease."

Retinal Cell and Gene Therapy Innovation Summit 2023

Dr. Barth's presentation at the Retinal Cell and Gene Therapy Innovation Summit will provide an update on Ascidian's lead program and the promise of RNA exon editing for patients with Stargardt disease and other *ABCA4*-related retinopathies.

Diseases caused by *ABCA4* loss of function represent an area of significant unmet need. More patients go blind from *ABCA4* retinopathy than any other genetic cause, and these diseases 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. By rewriting RNA, Ascidian's approach has the potential to treat patients with one dose of an RNA exon editing therapeutic delivered with a single AAV vector.

The Summit, jointly organized by the Foundation Fighting Blindness and the Oregon Health & Science University (OHSU) Casey Eye Institute, will bring together representatives from the biotechnology and pharmaceutical industries with physicians and scientists to discuss rapidly emerging ocular gene and cell therapies and strategize how to move the most advanced retinal disease therapy options forward. It will be held on Friday April 21, 2023 at the Marriott New Orleans Warehouse Arts District in New Orleans, LA.

ARVO 2023 Annual Meeting

At ARVO, Dr. Slomovic will present new six-month durability data from Ascidian's lead program targeting *ABCA4* retinopathies, which demonstrate the most efficient and durable RNA exon editing via trans-splicing ever reported in a large animal.

In addition, data demonstrating RNA exon editing in human retinal explants will also be reported. This is the first time RNA exon editing via trans-splicing has been demonstrated in human photoreceptors.

Presentation details are below.

Session: 414
Poster Title: *ABCA4* RNA exon editing *in vitro* and *in vivo*
Abstract Presentation Number: 3869
Date: Wednesday, April 26, 2023
Time: 10:30 a.m. to 12:30 p.m. CT
Location: The New Orleans Ernest N. Morial Convention Center, New Orleans, LA

The presentation materials from both sessions will be available upon request via the Publications & Presentations section of the Ascidian website following the live presentations.

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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 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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