Ascidian Therapeutics to Present New Data From Its Lead Program Targeting *ABCA4* Retinopathies at the ASGCT 2023 Annual Meeting

Latest six-month data from lead ABCA4 *program demonstrate efficient and durable production of full-length ABCA4 protein generated via RNA exon editing in non-human primate retina*

ABCA4 *program advancing to IND filing, as Ascidian progresses diversified pipeline of programs using its proprietary platform for rewriting RNA by exon editing*

BOSTON, May 9, 2023 – Ascidian Therapeutics, a biotechnology company focused on treating human diseases by rewriting RNA, announced today that Head of Research **Robert Bell, Ph.D.**, will present new data from its lead *ABCA4* program at the Twenty-Sixth Annual Meeting of the American Society of Gene and Cell Therapy (ASGCT).

The lead program targets *ABCA4*-related retinopathies, including Stargardt disease, and is currently in IND-enabling studies advancing towards clinical development.

The oral presentation, taking place at 9:00 a.m. PDT on May 20, will highlight Ascidian's groundbreaking RNA exon editing platform. Specifically, Dr. Bell will present new six-month data from Ascidian's lead *ABCA4* program that demonstrate the production 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 are the first to quantify therapeutically relevant levels of ABCA4 protein and represent the most efficient and durable RNA exon editing via trans-splicing ever demonstrated in large animals.

"These data could have therapeutically meaningful implications for patients with Stargardt disease, the most common form of inherited macular degeneration, as well as other diseases that are not addressable by today's gene therapy and gene editing technologies," said **Romesh Subramanian, Ph.D.**, President and Chief Executive Officer of Ascidian Therapeutics. "More than a quarter of a century has passed since the cause of Stargardt disease was discovered, and this is the first report quantifying and confirming therapeutically relevant levels of full-length ABCA4 protein produced after a single dose in a large animal. With durable editing now confirmed in non-human primates and replicated in human retinal explants, these findings add to the growing body of evidence supporting the potential of Ascidian's proprietary exon editing platform to transform the treatment of genetically defined diseases."

Dr. Bell's presentation at ASGCT will include data demonstrating *ABCA4* RNA exon editing in multiple model systems, including a cell line carrying *ABCA4* mutations, human retinal explants, and non-human primates at time points extending to six months. In addition to findings relating to full-length protein generation and RNA exon editing durability and efficiency, the presentation will also include data demonstrating the first successful RNA exon editing via trans-splicing in human photoreceptors *ex vivo*.

Presentation details are below.

Session: Gene Targeting and Gene Correction: CNS
Oral Presentation: Rewriting *ABCA4* RNA for the Treatment of Stargardt Disease
Date: Saturday May 20, 2023
Time: 9:00 a.m. – 9:15 a.m. PDT
Location: Room 515 AB, The Los Angeles Convention Center, Los Angeles, CA

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

# # #

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.

Company & Media Contacts:

Daniel Rosan
Chief Financial and Business Officer
outreach@ascidian-tx.com

Heather Shea
+1 617-286-2013
heather.shea@catalyticagency.com