



Ascidian Therapeutics Enters Collaboration with Roche for Discovery and Development of RNA Exon Editing Therapeutics Targeting Neurological Diseases

Ascidian to receive \$42 million in initial payment, and up to \$1.8 billion in research, clinical and commercial milestone payments, as well as commercial royalties

Per-target agreement enables Ascidian to pursue additional internal and collaborative programs within neurology and other therapeutic areas

Combines RNA Exon Editors with next generation CNS delivery capabilities of Roche to develop novel medicines for difficult to treat neurological diseases

BOSTON, June 18, 2024 – [Ascidian Therapeutics](#), a biotechnology company seeking to treat human diseases by rewriting RNA, today announced a research collaboration and licensing agreement with Roche (SIX: RO, ROG; OTCQX: RHHBY) for the discovery and development of RNA exon editing therapeutics targeting neurological diseases.

Ascidian's RNA exon editing platform is designed to advance the therapeutic possibilities of RNA medicine and treat diseases not addressed by today's gene editing technologies. The company designs and develops RNA exon editing therapeutics that edit RNA exons at the kilobase scale.

Under the agreement, Ascidian will provide Roche exclusive, target-specific rights to Ascidian's RNA exon editing technology for undisclosed neurological targets. Ascidian will conduct discovery and certain preclinical activities in collaboration with Roche, and Roche will be responsible for certain preclinical activities, and further clinical development, manufacturing, and commercialization. Ascidian will receive an initial payment of \$42 million and is eligible to receive up to \$1.8 billion in research, clinical, and commercial milestone payments, as well as royalties on commercial sales worldwide. Based on the terms of the agreement, Ascidian is free to develop programs against other neurological targets internally or with other collaborators.

"Roche is known and respected worldwide for their expertise in complex neurological diseases, and I am proud of the scientific rigor and quality of the work done at Ascidian that has led to this partnership," said **Michael Ehlers, M.D., Ph.D.**, President and Chief Executive Officer of Ascidian Therapeutics. "The potential of treating disease by large-scale exon editing of RNA is vast. We look forward to working with the Roche team to develop first-in-class RNA exon editing medicines for multiple neurological diseases, with a mission and passion to relieve suffering and improve lives."

"Our partnership with Ascidian is an opportunity to harness advanced RNA exon editing technology, which has the potential to deliver transformative one-time therapeutics by editing multiple whole exons at the RNA level with a single treatment," said **James Sabry, M.D., Ph.D.**, Global Head of Pharma Partnering at Roche.

Ascidian's platform enables targeting of large genes and genes with high mutational variance while maintaining native gene expression patterns and levels. By rewriting RNA, Ascidian's exon editing technology is designed to provide the durability of gene therapy, while sharply reducing risks associated with direct DNA editing and gene replacement.

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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. Earlier this year, Ascidian announced U.S. FDA IND clearance for the first-ever RNA exon editing candidate, ACDN-01, which targets Stargardt disease and other *ABCA4* retinopathies. Ascidian is currently executing the Phase 1/2 STELLAR clinical trial to evaluate the safety and efficacy of ACDN-01. For more information, visit www.ascidian.com.

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