Arrowhead Pharmaceuticals and Novartis Enter into a Global License and Collaboration Agreement

September 2, 2025

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- Novartis will receive an exclusive worldwide license to ARO-SNCA, Arrowhead's preclinical stage siRNA therapy for the treatment of synucleinopathies, such as Parkinson's Disease, plus additional collaboration targets

PASADENA, Calif.--(BUSINESS WIRE)--Sep. 2, 2025-- Arrowhead Pharmaceuticals, Inc. (NASDAQ: ARWR) today announced a global licensing and collaboration agreement with Novartis for ARO-SNCA, Arrowhead's preclinical stage siRNA therapy against alpha-synuclein for the treatment of synucleinopathies, such as Parkinson's Disease, and for other additional collaboration targets that will utilize Arrowhead's proprietary Targeted RNAi Molecule (TRiM™) platform. Upon closing, Arrowhead will receive $200 million as an upfront payment and is eligible to receive up to $2 billion in potential milestone payments plus royalties on commercial sales.

"With a robust neuroscience pipeline and clear commitment to neurodegenerative diseases and genetic medicines, Novartis is a compelling partner for Arrowhead in the CNS space," said Christopher Anzalone, Ph.D., President and CEO at Arrowhead. "Our TRiM™ platform has generated impressive preclinical results demonstrating delivery to CNS, including distribution to deep brain regions, after subcutaneous administration. The potential translation of these results in upcoming clinical trials would represent an important leap forward for neurodegenerative diseases and gene targets in the CNS that have been historically difficult to address. We look forward to working with Novartis to bring ARO-SNCA for the treatment of synucleinopathies, such as Parkinson's Disease, into clinical trials as soon as possible and to collaborate on additional programs in the future."

Fiona Marshall, Ph.D., President of Biomedical Research at Novartis added, "Neurodegenerative conditions such as Parkinson's Disease affect millions of patients worldwide. Novartis aspires to transform the lives of patients and families living with these diseases, by advancing medicines that significantly alter the course of the disease. We believe that one way to effectively target core drivers in Parkinson's and other neurodegenerative diseases requires completely novel approaches to deliver RNA medicines to the brain. We see Arrowhead's TRiM™ technology as having great potential to achieve the type of widespread and effective delivery in key brain structures that will be necessary to see the full benefit of RNA medicines in neurodegeneration."

Summary of License and Collaboration Agreement

Under the terms of the agreement, Novartis will receive an exclusive worldwide license to research, develop, manufacture, and commercialize ARO-SNCA, a preclinical stage program that utilizes Arrowhead's TRiM™ platform for subcutaneous administration and delivery to the CNS designed to target the gene that encodes the alpha-synuclein protein as a potential treatment for patients with Parkinson's Disease, and other synucleinopathies. Novartis will select additional collaboration targets outside of Arrowhead's current pipeline to be developed using the TRiM™ platform.

For all licensed programs under the agreement, Arrowhead will conduct and complete preclinical research activities necessary to enable a clinical trial application (CTA) filing. Novartis will then assume sole control over development, manufacturing, medical affairs, and commercialization activities.

Summary Financial Terms

Upon closing, Novartis will make a $200 million upfront payment to Arrowhead. Arrowhead is also eligible to receive development, regulatory, and sales milestone payments of up to $2 billion. Arrowhead is further eligible to receive tiered royalties on commercial sales up to the low double digits.

The transaction is expected to close in the second half of 2025, subject to the expiration or termination of the waiting period under the Hart-Scott-Rodino Antitrust Improvements Act of 1976 and other customary conditions.

Gibson, Dunn & Crutcher LLP is serving as legal advisor to Arrowhead.

About Arrowhead Pharmaceuticals

Arrowhead Pharmaceuticals develops medicines that treat intractable diseases by silencing the genes that cause them. Using a broad portfolio of RNA chemistries and efficient modes of delivery, Arrowhead therapies trigger the RNA interference mechanism to induce rapid, deep, and durable knockdown of target genes. RNA interference, or RNAi, is a mechanism present in living cells that inhibits the expression of a specific gene, thereby affecting the production of a specific protein. Arrowhead's RNAi-based therapeutics leverage this natural pathway of gene silencing.

For more information, please visit www.arrowheadpharma.com, or follow us on X (formerly Twitter) at @ArrowheadPharma, LinkedIn, Facebook, and Instagram. To be added to the Company's email list and receive news directly, please visit http://ir.arrowheadpharma.com/email-alerts.

Safe Harbor Statement under the Private Securities Litigation Reform Act:

This news release contains forward-looking statements within the meaning of the "safe harbor" provisions of the Private Securities Litigation Reform Act of 1995. Any statements contained in this release except for historical information may be deemed to be forward-looking statements. Without limiting the generality of the foregoing, words such as "may," "will," "expect," "believe," "anticipate," "hope," "intend," "plan," "project," "could," "estimate," "continue," "target," "forecast" or "continue" or the negative of these words or other variations thereof or comparable terminology are intended to identify such forward-looking statements. In addition, any statements that refer to projections of our future financial performance, trends in our business, expectations for our product pipeline or product candidates, including anticipated regulatory submissions and clinical program results, prospects or benefits of our collaborations with other companies, or other characterizations of future events or circumstances are forward-looking statements. These forward-looking statements include, but are not limited to, statements about the initiation, timing, progress and results of our preclinical studies and clinical trials, and our research and development programs; our expectations regarding the potential benefits of the partnership, licensing and/or collaboration arrangements and other strategic arrangements and transactions we have entered into or may enter into in the future; our beliefs and expectations regarding milestone, royalty or other payments that could be due to or from third parties under existing agreements; and

our estimates regarding future revenues, research and development expenses, capital requirements and payments to third parties. These statements are based upon our current expectations and speak only as of the date hereof. Our actual results may differ materially and adversely from those expressed in any forward-looking statements as a result of numerous factors and uncertainties, including the impact of the ongoing COVID-19 pandemic on our business, the safety and efficacy of our product candidates, decisions of regulatory authorities and the timing thereof, the duration and impact of regulatory delays in our clinical programs, our ability to finance our operations, the likelihood and timing of the receipt of future milestone and licensing fees, the future success of our scientific studies, our ability to successfully develop and commercialize drug candidates, the timing for starting and completing clinical trials, rapid technological change in our markets, the enforcement of our intellectual property rights, and the other risks and uncertainties described in our most recent Annual Report on Form 10-K, subsequent Quarterly Reports on Form 10-Q and other documents filed with the Securities and Exchange Commission from time to time. We assume no obligation to update or revise forward-looking statements to reflect new events or circumstances.

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