Arrowhead Pharmaceuticals Earns $100 Million Milestone from Sarepta Therapeutics

July 28, 2025

- Milestone earned upon reaching first enrollment target in a Phase 1/2 study of ARO-DM1 for the treatment of type 1 myotonic dystrophy

PASADENA, Calif.--(BUSINESS WIRE)--Jul. 28, 2025-- Arrowhead Pharmaceuticals, Inc. (NASDAQ: ARWR) today announced that it has earned a $100 million milestone payment from Sarepta Therapeutics (NASDAQ: SRPT). The milestone was triggered when Arrowhead reached the first of two prespecified enrollment targets and subsequent authorization to dose escalate in a Phase 1/2 clinical study of ARO-DM1, an investigational RNA interference (RNAi) therapeutic for the treatment of type 1 myotonic dystrophy (DM1), the most common adult-onset muscular dystrophy. Arrowhead currently expects to achieve the second enrollment target by the end of 2025, which would trigger an additional $200 million milestone payment from Sarepta.

In accordance with the license and collaboration agreement, Arrowhead expects to receive this payment within 60 days.

About the Arrowhead-Sarepta Agreement

Arrowhead and Sarepta signed a global licensing and collaboration agreement in November 2024, which closed in February 2025, whereby Sarepta received rights to multiple investigational treatments that leverage Arrowhead's leading Targeted RNAi Molecule (TRiM ^{TM}) platform. The agreement covers multiple clinical and preclinical programs in rare, genetic diseases of the muscle, central nervous system, and the lungs, and also allows Sarepta to select up to six new targets for Arrowhead to conduct discovery and preclinical development activities in areas complementary to Sarepta's leadership in precision genetic medicine for rare diseases.

Summary Financial Terms

Upon closing, Arrowhead received a $500 million upfront payment and $325 million through the purchase by Sarepta of Arrowhead common stock priced at $27.25 per share, representing a 35% premium to the 30-day volume weighted average price (VWAP) when the agreement was signed. Arrowhead will also receive $250 million to be paid in annual installments of $50 million over five years, and has the potential to receive an additional $300 million in near-term milestone payments associated with the continued enrollment of certain cohorts of a Phase 1/2 study of ARO-DM1, $100 million of which has now been earned.

Arrowhead is also eligible to receive further development milestone payments of between $110 million and $180 million per program, sales milestone payments of between $500 million and $700 million per program, and tiered royalties on commercial sales up to the low double digits.

Summary of Programs Under the License and Collaboration Agreement

Clinical Stage

- ARO-DUX4, which is designed to target the gene that encodes the DUX4 protein as a potential treatment for patients with facioscapulohumeral muscular dystrophy type 1, currently dosing patients in a Phase 1/2 clinical study.
- ARO-DM1, which is designed to reduce expression of the dystrophia myotonica protein kinase (DMPK), gene in skeletal muscle as a potential treatment for patients with type 1 myotonic dystrophy, currently dosing patients in a Phase 1/2 clinical study.
- ARO-MMP7, which is designed to reduce expression of matrix metalloproteinase 7 (MMP7) in the lung as a potential treatment for idiopathic pulmonary fibrosis, currently dosing patients in a Phase 1/2 clinical study.
- ARO-ATXN2, which is designed to silence expression of the toxic ATXN2 protein in the CNS as a potential treatment for spinocerebellar ataxia 2 (SCA2), currently in a Phase 1/2 study that is open for enrollment.

Preclinical Stage

- ARO-HTT for patients with Huntington's disease, expected to be CTA-ready in 2025
- ARO-ATXN1 for patients with spinocerebellar ataxia 1 (SCA1) expected to be CTA-ready in 2026
- ARO-ATXN3 for patients with spinocerebellar ataxia 3 (SCA3) expected to be CTA-ready in 2026

Discovery

During the five-year term, Sarepta can propose up to six new CNS or muscle targets for which Arrowhead will perform discovery and preclinical development. Sarepta would then receive an exclusive license to those programs and would be responsible for subsequent clinical development and commercialization.

Drug Manufacturing

Under the terms of the agreement, Arrowhead will manufacture clinical drug supply for all programs arising out of the license and collaboration, and commercial drug product for the four programs currently in clinical trials.

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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