Arrowhead Pharmaceuticals Announces Global License and Collaboration Agreement with Sarepta Therapeutics for Multiple Clinical and Preclinical Programs

November 26, 2024

- Upon closing, Arrowhead will receive $825 million immediately, including an upfront payment and an equity investment at a 35% premium, and will receive an additional $250 million paid over five years

- Arrowhead has potential to receive an additional $300 million in near-term clinical trial enrollment-related milestone payments and is eligible for future potential milestone payments up to $10 billion and royalties on sales

- Sarepta to receive investigational treatments that leverage Arrowhead's leading Targeted RNAi Molecule platform

- Arrowhead to discuss this agreement during the company's 2024 fiscal year end results conference call today, November 26, 2024, at 4:30 p.m. ET

PASADENA, Calif.--(BUSINESS WIRE)--Nov. 26, 2024-- Arrowhead Pharmaceuticals, Inc. (NASDAQ: ARWR) today announced a global licensing and collaboration agreement with Sarepta Therapeutics (NASDAQ: SRPT). Upon closing, Arrowhead will receive $825 million, consisting of $500 million cash and $325 million as an equity investment priced at a 35% premium. Arrowhead also receives $250 million to be paid in equal installments over five years and is eligible to receive an additional $300 million in near-term payments, which Arrowhead is on track to achieve during the next 12 months. Additionally, Arrowhead is eligible to receive royalties on commercial sales and up to approximately $10 billion in future potential milestone payments.

The agreement covers multiple clinical and preclinical programs in rare, genetic diseases of the muscle, central nervous system (CNS), and the lungs, as well as 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, which can utilize Arrowhead's proprietary and differentiated Targeted RNAi Molecule (TRiM™) platform.

"For Arrowhead, this strategic transaction accomplishes several important goals that we see as critical to our success as we move from a development stage company operating in several therapeutic areas to a more focused commercial stage company over the coming years. It provides us with a transformational amount of capital immediately and the potential for significant non-dilutive cash throughout the duration of the collaboration. We estimate that this transaction extends Arrowhead's cash runway into 2028 and potentially through multiple new drug launches, including wholly owned and partnered programs. We now turn our focus as a company to launching investigational plozasiran for the treatment of familial chylomicronemia syndrome potentially in 2025, pending FDA review and approval, which would be our first commercial product. We are also prioritizing further development of additional pipeline programs which would be complementary to plozasiran from a clinical, regulatory, and commercial perspective," said Christopher Anzalone, Ph.D., president and CEO at Arrowhead. "With this agreement we also welcome the Sarepta team as new Arrowhead collaboration partners who bring a wealth of clinical, regulatory, and commercial expertise in key areas outside of our cardiometabolic focus. We have a very efficient drug discovery engine that continues to generate many promising programs and we have great confidence in Sarepta's ability to take the next steps to advance and commercialize multiple Arrowhead-discovered drug candidates, which we believe have the potential to be best-in-class."

At the close of the agreement, Doug Ingram, president and CEO of Sarepta, will be appointed to the Arrowhead Board of Directors. He is an experienced biotech and pharma executive and has led Sarepta as they advanced multiple investigational medicines through the clinical and regulatory process, built a commercial organization from the ground up, launched multiple drugs, and moved the company toward profitability. His experience and guidance will be valuable to Arrowhead as the Company seeks the same transition.

Mr. Ingram added, "The agreement marks the start of a synergistic relationship with Arrowhead's leading siRNA technology and Sarepta's proven success in bringing rare disease treatments to patients. Together, we will expedite the development of Arrowhead's promising scientific approach and bring best-in-class treatments to patients with devastating rare diseases where treatment options are limited or do not exist. I am honored to serve on the Arrowhead Board of Directors and to help Arrowhead advance its extraordinary science for the benefit of patients around the world."

Summary Financial Terms

Upon closing, Arrowhead will receive a $500 million upfront payment and $325 million through the purchase by Sarepta of Arrowhead common stock priced at a 35% premium to the 30-day volume weighted average price. Arrowhead will also receive $250 million to be paid in annual installments of $50 million over 5 years. Arrowhead also has the potential to receive $300 million in near-term payments associated with the continued enrollment of certain cohorts of a Phase 1/2 study, which Arrowhead is on track to achieve during the next 12 months.

Arrowhead is eligible to receive development milestone payments of between $110 million and $410 million per program and sales milestone payments of between $500 million and $700 million per program. Arrowhead is also eligible to receive tiered royalties on commercial sales up to the low double digits.

Summary of Programs under 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, or 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, or 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 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 be responsible for clinical development and commercialization.

Drug Manufacturing

Under 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.

The transaction is expected to close in early 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.

Webcast and Conference Call and Details

Arrowhead will discuss this agreement during its 2024 fiscal year-end results conference call today, November 26, 2024, at 4:30 p.m. ET.

Investors may access a live audio webcast on the Events and Presentations page under the Investors section of the Arrowhead website. A replay of the webcast will be available approximately two hours after the conclusion of the call.

For analysts that wish to participate in the conference call, please register at https://register.vevent.com/register/BIc060a13ae8224934aac09d48abe7b2f6. Once registered, you will receive the dial-in number and a personalized PIN code that will be required to access the call.

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.

Source: Arrowhead Pharmaceuticals, Inc.

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Source: Arrowhead Pharmaceuticals, Inc.