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

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– ***Sarepta obtains exclusive worldwide licenses to four clinical-stage and three preclinical-stage programs in muscle, central nervous system, and rare pulmonary disorders, including potential best-in-class siRNA-based treatments for DM1 and FSHD***

– ***Additionally, Arrowhead and Sarepta have entered into a discovery partnership pursuant to which Sarepta will nominate, and Arrowhead will deliver, IND-ready constructs for six targets across skeletal muscle, cardiac, and CNS***

– ***Investigational treatments leverage Arrowhead's leading Targeted RNAi Molecule (TRiM ^{TM}) platform, capable of deep and durable target-gene knockdown***

– ***Upon closing, Arrowhead to receive $500 million in an upfront payment and $325 million equity investment at a 35% premium, plus additional future milestone payments and royalties***

– ***Separately, Sarepta's Board of Directors has approved a share repurchase authorization of up to $500 million***

– ***Sarepta to host an investor conference call on Tuesday, Nov. 26, 2024, at 8:30 a.m. ET***

CAMBRIDGE, Mass.--(BUSINESS WIRE)--Nov. 26, 2024-- Sarepta Therapeutics, Inc. (NASDAQ:SRPT), the leader in precision genetic medicine for rare diseases, today announced an exclusive global licensing and collaboration agreement with Arrowhead Pharmaceuticals, Inc. (NASDAQ: ARWR). Sarepta will obtain exclusive global rights to multiple clinical, preclinical, and discovery-stage programs for rare, genetic diseases of the muscle, central nervous system (CNS), and the lungs.

The agreement will add meaningfully to Sarepta's mid- and early-stage pipeline, complementing the Company's existing leadership in Duchenne muscular dystrophy and limb-girdle muscular dystrophies and gene therapy, while adding new indications and expanding into adjacent therapeutic areas. In addition, Doug Ingram, president and chief executive officer, Sarepta, will be appointed to Arrowhead's Board of Directors.

The clinical-stage programs covered under the agreement include:

- **ARO-DUX4:** designed to reduce the production of human double homeobox 4 (DUX4) protein in skeletal muscle; currently in a Phase 1/2 clinical study for the treatment of facioscapulohumeral muscular dystrophy (FSHD)
- **ARO-DM1:** designed to target and suppress myotonic dystrophy protein kinase (DMPK) in skeletal muscle; Phase 1/2 clinical study for myotonic dystrophy type 1 (DM1)
- **ARO-MMP2**: designed to reduce expression of matrix metalloproteinase 7 (MMP7) in pulmonary epithelial cells; Phase 1/ clinical study for idiopathic pulmonary fibrosis (IPF)
- **ARO-ATXN2** : designed to target the ataxin-2 protein (ATXN2) in the CNS; expected to begin Phase 1/2 clinical study for spinocerebellar ataxia 2 (SCA2) by the end of 2024

The clinical programs use Arrowhead's proprietary Targeted RNAi Molecule (TRiM ^{TM}) platform, which is designed to deliver siRNA to multiple tissue and cell types throughout the body to initiate the RNA interference mechanism and induce rapid and durable knockdown of target genes.

The preclinical programs covered under the agreement will leverage Arrowhead's TRiM CNS delivery platform designed for subcutaneous administration and include:

- **ARO-ATXN1**: designed to target the ataxin-1 protein (ATXN1) for SCA1
- **ARO-ATXN3**: designed to target the ataxin-3 protein (ATXN3) for SCA3
- **ARO-HTT**: designed to target huntingtin (HTT), a gene linked to Huntington's disease

Additionally, Sarepta and Arrowhead have entered into a discovery collaboration for up to six additional muscle, cardiac, and/or CNS targets, using Arrowhead's novel delivery technologies. As part of the collaboration, Sarepta has an exclusive license to Arrowhead's technology to develop therapeutics against a broad range of skeletal muscle gene targets.

"With the launch of Elevidys going exceedingly well, this broad siRNA collaboration with Arrowhead provides a synergistic platform to complement Sarepta's gene therapy and gene editing engine. Through a strategic deployment of capital, we are able to access Arrowhead's leading RNAi platform and will work to rapidly advance new treatments for devastating genetic diseases where there is significant unmet need. The agreement affords multiple potential blockbuster opportunities, serves our strategic priorities for the remainder of the decade and beyond, and diversifies our business model across one-time therapies and chronic treatments allowing for long-term growth and success. Given the strength of our performance and ability to generate substantial cash to invest in our business over the next several years, Sarepta's Board of Directors has approved a $500 million share repurchase program as part of our overall capital allocation strategy," said Mr. Ingram. "We look forward to embarking on this partnership with Arrowhead, having been impressed with their scientific capabilities in developing a potentially best-in-class approach to siRNA and the quality of the team they have built. Over the course of the next 12-18 months, we expect to share multiple data readouts from across our pipeline."

“We 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 see our TRiM platform as a broad and elegant solution for delivery of siRNA to multiple cell types throughout the body. We also 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,” said Chris Anzalone, Ph.D., president and CEO at Arrowhead. “At the close of this agreement, Doug Ingram will be appointed to the Arrowhead board of directors. He 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 as Arrowhead seeks the same transition.”

“Robust and compelling early data from Arrowhead’s differentiated siRNA approach platform suggests potentially best-in-class treatments that will profoundly improve the lives of those with rare, genetic diseases,” said Louise Rodino-Klapac, Ph.D., chief scientific officer and head of research and development, Sarepta. “The targeted ligand approach, combined with Arrowhead’s clinically validated siRNA chemistry, suggests the potential for deep and durable knockdown of proteins that are over-expressed in these conditions. Arrowhead’s innovative approach to cross the blood brain barrier with subcutaneous dosing represents a potential paradigm shift for the CNS preclinical and discovery programs as part of this collaboration.”

Summary of Financial Terms

Under the terms of the agreement, Sarepta will pay Arrowhead an upfront payment of $500 million and an equity investment of $325 million in Arrowhead common stock, priced at a 35% premium to the 30-day volume weighted average price prior to the announcement of the agreement. Arrowhead will also receive $250 million to be paid in annual installments of $50 million over five years, in addition to being eligible for future milestone payments and royalties. Arrowhead will be responsible for Phase 1/2 trials currently underway, and clinical stage programs will transition to Sarepta no later than the completion of current trials. Preclinical assets will transition to Sarepta upon completion of IND-enabling activities by Arrowhead.

The deal will be funded with cash on hand and Sarepta does not plan to raise additional capital via the debt or equity capital markets. The transaction is expected to close 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.

Ropes & Gray LLP is serving as legal advisor to Sarepta.

Share Repurchase Program

Sarepta’s Board of Directors has also approved a share repurchase program of up to $500 million of the Company’s outstanding common stock over the next 18 months.

The timing of any stock repurchases will be based upon the continuing analysis of market, financial, and other factors by the Company’s management. Repurchases under the authorized stock repurchase program may be made using a variety of methods, which may include, but are not limited to, open market purchases, privately negotiated transactions, accelerated share repurchase agreements or purchases pursuant to a Rule 10b5-1 plan under the Securities Exchange Act of 1934, as amended. The authorized stock repurchase program may be suspended, delayed or discontinued at any time.

Sarepta Investor Call Details

At 8:30 a.m. ET on Nov. 26, 2024, Sarepta will host a conference call and webcast to discuss this agreement.

The event will be webcast live under the investor relations section of Sarepta’s website at https://investorrelations.sarepta.com/events-presentations and following the event a replay will be archived there for one year. Interested parties participating by phone will need to register using this online form. After registering for dial-in details, all phone participants will receive an auto-generated e-mail containing a link to the dial-in number along with a personal PIN number to use to access the event by phone.

About Sarepta Therapeutics

Sarepta is on an urgent mission: engineer precision genetic medicine for rare diseases that devastate lives and cut futures short. We hold leadership positions in Duchenne muscular dystrophy (DMD) and limb-girdle muscular dystrophies (LGMDs), and we currently have more than 40 programs in various stages of development. Our vast pipeline is driven by our multi-platform Precision Genetic Medicine Engine in gene therapy, RNA and gene editing. For more information, please visit www.sarepta.com or follow us on LinkedIn, X, Instagram and Facebook.

Sarepta Forward-Looking Statements

This press release contains “forward-looking statements.” Any statements contained in this press release that are not statements of historical fact may be deemed to be forward-looking statements. Words such as “believes,” “anticipates,” “plans,” “expects,” “will,” “intends,” “potential,” “possible” and similar expressions are intended to identify forward-looking statements. These forward-looking statements include but are not limited to statements regarding the closing of the transaction and the equity investment; Sarepta’s payment of regulatory and sales milestones, and royalty payments to Arrowhead pursuant to the agreement; the expected targets of the clinical and pre-clinical programs licensed pursuant to the agreement; ongoing development of therapeutics against a broad range of skeletal muscle gene targets by Sarepta and Arrowhead; Arrowhead’s potentially best-in-class approach to siRNA; the expected timing of future data readouts; and statements about the amount and timing of repurchases under Sarepta’s share purchase program and the methods to execute such repurchases.

These forward-looking statements involve risks and uncertainties, many of which are beyond Sarepta's control. Known risk factors include, among others, market conditions, the expected benefits and opportunities related to the collaboration agreement may not be realized or may take longer to realize than expected due to a variety of reasons, including any inability of the parties to perform their commitments and obligations under the agreement, challenges and uncertainties inherent in product research and development and manufacturing limitations; success in preclinical testing and early clinical trials, especially if based on a small patient sample, does not ensure that later clinical trials will be successful, and early results from a clinical trial do not necessarily predict final results; Sarepta may not be able to execute on its business plans, including meeting its expected or planned regulatory milestones and timelines, research and clinical development plans, for various reasons, some of which may be outside of Sarepta’s control, including possible limitations of company financial and other resources; and those risks identified under the heading “Risk Factors” in

Sarepta's most recent Quarterly Report on Form 10-Q for the quarter ended September 30, 2024, as well as other SEC filings made by the Company which you are encouraged to review.

Any of the foregoing risks could materially and adversely affect Sarepta's business, results of operations and the trading price of Sarepta's common stock. We caution investors not to place considerable reliance on the forward-looking statements contained in this press release. Sarepta does not undertake any obligation to publicly update its forward-looking statements based on events or circumstances after the date hereof, except as required by law.

Source: Sarepta Therapeutics, Inc.

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