Arrowhead Pharmaceuticals to Host R&D Day on Pipeline of RNAi Therapeutics

April 12, 2023

- Event to include presentations on cardiometabolic, pulmonary, and newly announced central nervous system pipeline programs

PASADENA, Calif.--(BUSINESS WIRE)--Apr. 12, 2023-- Arrowhead Pharmaceuticals Inc. (NASDAQ: ARWR) today announced that it will host a Research & Development (R&D) Day on June 1, 2023, beginning at 9 a.m. ET in New York City to discuss progress towards the company's "20 in 25" goal to grow its pipeline of RNAi therapeutics that leverage the proprietary Targeted RNAi Molecule (TRiM™) platform to a total of 20 clinical stage or marketed products in the year 2025.

Arrowhead also announced that company has expanded its TRiM™ platform to include an optimized intrathecal administration for central nervous system (CNS) delivery with good distribution throughout the brain and in all relevant brain cell types. The first development candidate to utilize this new delivery platform, ARO-SOD1, is on track for a clinical trial application (CTA) filing in the third quarter of 2023 to begin clinical studies.

ARO-SOD1 is designed to reduce expression of superoxide dismutase 1 (SOD1) in the CNS as a potential treatment for patients with amyotrophic lateral sclerosis (ALS) caused by SOD1 mutations. ARO-SOD1 was highly active against its target with a long duration of effect in multiple preclinical models suggesting that it may be administered quarterly or less frequently. In preclinical studies, ARO-SOD1 achieved 95% spinal cord tissue mRNA knockdown after a single intrathecal dose in human SOD1 transgenic rats and maintained greater than 80% spinal cord tissue mRNA knockdown three months after a single intrathecal dose in non-human primates.

The R&D Day will feature presentations by three key opinion leaders: Ira Goldberg, M.D. (NYU Langone Medical Center), who will discuss the unmet medical need in treating patients with hypertriglyceridemia and mixed dyslipidemia in atherosclerotic cardiovascular disease (ASCVD); Matthias Salathe, M.D. (University of Kansas Medical Center), who will discuss the muco-obstructive and inflammatory pulmonary disease landscape; and Michael Benatar, M.D., Ph.D. (University of Miami Miller School of Medicine), who will discuss ALS caused by SOD1 mutations. Drs. Goldberg, Salathe, and Benatar will be available to answer questions following their presentations.

The R&D Day will also feature presentations by the Arrowhead team who will provide updates on multiple programs, including:

Cardiometabolic programs:

- ARO-APOC3 for the treatment of patients with familial chylomicronemia syndrome, severe hypertriglyceridemia, and mixed dyslipidemia in ASCVD. Currently in a Phase 3 study and multiple Phase 2b studies.
- ARO-ANG3 for the treatment of patients with familial hypercholesterolemia. Currently in multiple Phase 2b studies.

Pulmonary programs:

- ARO-RAGE for the treatment of patients with asthma. Currently in a Phase 1/2 study.
- ARO-MUC5AC for the treatment of patients with muco-obstructive pulmonary diseases. Currently in a Phase 1/2 study.

Central nervous system:

- Development of the TRiM™ platform for CNS delivery.
- ARO-SOD1 for treatment of patients with ALS caused by SOD1 mutations. A CTA filing is planned for the third quarter of 2023.

Additional liver targeted programs:

- ARO-C3 for treatment of patients with various complement mediated diseases. Currently in a Phase 1/2 study.
- ARO-PNPLA3 for treatment of patients with non-alcoholic steatohepatitis. Currently in a Phase 1/2 study.

This event is intended for institutional investors, sell-side research analysts, and business development professionals only. Please RSVP in advance if you plan to attend, as space is limited. To reserve a seat, please click here to register.

A copy of the presentation materials and webcast links may be accessed on the Events and Presentations page under the Investors section of the Arrowhead website.

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 Twitter @ArrowheadPharma. 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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