Arrowhead Hosts R&D Day Highlighting Its Pipeline of RNAi Therapeutics

June 1, 2023

- New Clinical Data on ARO-RAGE Show Continued Dose Response with Single Inhaled Dose of 184 mg Achieving Mean Knockdown of 90% and Max of 95%

- Adipose Delivery Platform Achieved Single Dose Target Gene Silencing of Greater than 90% with Six Months of Duration in Non-human Primates

- Improved Hepatic Dimer Platform Achieves Equivalent or Better Knockdown of Two Target Genes with Longer Duration than Monomer Mixture in Non-human Primates

- TRiM™ Platform Now has Potential to Address Multiple Cell Types Including Liver, Solid Tumors, Lung, Central Nervous System, Skeletal Muscle, and Adipose

PASADENA, Calif.--(BUSINESS WIRE)--Jun. 1, 2023-- Arrowhead Pharmaceuticals Inc. (NASDAQ: ARWR) is hosting a Research & Development (R&D) Day today beginning at 9:00 a.m. ET 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.

A webcast link may be accessed on the Events and Presentations page under the Investors section of the Arrowhead website and the presentation materials will be posted following conclusion of the event.

Christopher Anzalone, Ph.D., President and CEO at Arrowhead, said: "In 2017 we had optimized the TRiM™ platform for hepatocyte delivery, but we had yet to advance any candidates into clinical studies. By the end of 2023, just 6 years later, we expect to have advanced 18 drug candidates into clinical studies addressing multiple cell types, including liver, solid tumor, pulmonary, CNS, and skeletal muscle, with three candidates currently in Phase 3 studies. This has been an enormously productive period for Arrowhead, which we intend to continue as we make progress towards our 20 in 25 goal to have 20 clinical stage or marketed products that utilize the TRiM™ platform in the year 2025. We are excited about the progress we've achieved to date and by what lies ahead, as we continue to expand the platform into systemic delivery to the CNS that crosses the blood brain barrier, delivery to adipose tissue, and delivery of dimers that can knock down two separate genes."

The R&D Day will feature presentations by three key opinion leaders: Michael Benatar, M.D., Ph.D. (University of Miami Miller School of Medicine), who will discuss amyotrophic lateral sclerosis (ALS) caused by superoxide dismutase 1 (SOD1) mutations; Matthias Salathe, M.D. (University of Kansas Medical Center), who will discuss the muco-obstructive and inflammatory pulmonary disease landscape; and 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). Drs. Benatar, Salathe, and Goldberg 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, in the following order:

Central nervous system (CNS):

- Development of the TRiM™ platform for intrathecal administration for CNS delivery demonstrates 90-95% dose-dependent mRNA knockdown in disease-relevant spinal cord and cortex brain regions in non-human primates.
- ARO-SOD1 for treatment of patients with ALS caused by SOD1 mutations. A CTA filing is planned for the third quarter of 2023.

TRiM™ platform expansion progress:

- TRiM™ platform for systemic administration delivered siRNA across the blood brain barrier and achieved knockdown in non-human primates in all brain regions, including deep brain.
- TRiM™ platform for adipose tissue delivery achieved up to 98% knockdown and maintained greater than 85% knockdown over 31 weeks.
- TRiM™ dimer platform for delivery to hepatocytes achieved equivalent or better knockdown of two separate target genes with longer duration than monomer mixture.

Pulmonary programs:

- ARO-RAGE for the treatment of patients with asthma is currently in a Phase 1/2 study.
    - New clinical data demonstrate continued dose response with single inhaled dose of 184 mg achieving mean knockdown of 90% and maximum knockdown of 95%.
    - New clinical data also show ARO-RAGE achieved serum sRAGE reductions in asthma patients consistent with effects seen in healthy volunteers at the 44 mg dose, the only dose level currently available in asthma patient cohorts.
- ARO-MUC5AC for the treatment of patients with muco-obstructive pulmonary diseases is currently in a Phase 1/2 study.
- ARO-MMP7 for the treatment of patients with idiopathic pulmonary fibrosis is currently in a Phase 1/2 study.

Early clinical stage liver targeted programs:

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

Cardiometabolic programs:

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

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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