Arrowhead Pharmaceuticals Initiates Phase 1/2a Studies for Two Pulmonary Candidates ARO-MUC5AC and ARO-RAGE

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PASADENA, Calif.--(BUSINESS WIRE)--Jul. 5, 2022-- Arrowhead Pharmaceuticals Inc. (NASDAQ: ARWR) today announced that it has dosed the first subjects in two Phase 1/2a clinical trials of ARO-MUC5AC and ARO-RAGE, the company's investigational RNA interference (RNAi) therapeutics designed to reduce production the of mucin 5AC (MUC5AC) and the receptor for advanced glycation end products (RAGE), respectively, as potential treatments for various muco-obstructive and inflammatory pulmonary diseases.

"Initiating clinical studies for ARO-MUC5AC and ARO-RAGE, Arrowhead's next generation pulmonary targeted RNAi therapeutic candidates, is a significant milestone for the company," said Chris Anzalone, Ph.D., president and chief executive officer of Arrowhead. "These are potentially important new medicines that address targets that have been difficult to drug using other modalities and are designed to treat muco-obstructive and inflammatory lung diseases in fundamentally new ways."

AROMUC5AC-1001 (NCT05292950) is a Phase 1/2a, randomized, double-blinded, placebo-controlled study in up to 42 normal healthy volunteers (NHV) and up to 16 patients with moderate-to-severe asthma. The single ascending dose portion of the study includes 3 sequentially enrolled NHV cohorts with escalating single-dose levels. The multiple ascending dose portion of the study includes 3 NHV cohorts and 2 asthma patient cohorts. The objectives of the study include the assessment of safety and tolerability, pharmacokinetics, and pharmacodynamics of ARO-MUC5AC in NHVs and patients with asthma.

ARORAGE-1001 (NCT05276570) is a Phase 1/2a, randomized, double-blinded, placebo-controlled study in up to 64 NHVs and up to 16 patients with mild-to-moderate asthma. The single ascending dose portion of the study includes 4 sequentially enrolled NHV cohorts and one optional cohort treated with escalating single-dose levels. The multiple ascending dose portion of the study includes 4 NHV cohorts and 2 asthma patient cohorts. The objectives of the study include the assessment of safety and tolerability, pharmacokinetics, and pharmacodynamics of ARO-RAGE in NHVs and patients with asthma.

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," or "continue" 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 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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