Arrowhead Files for Regulatory Clearance to Initiate Phase 1/2a Study of ARO-RAGE for Treatment of Asthma

March 15, 2022

PASADENA, Calif.--(BUSINESS WIRE)--Mar. 15, 2022-- Arrowhead Pharmaceuticals Inc. (NASDAQ: ARWR) today announced that it has filed an application for clearance to initiate a Phase 1/2a clinical trial of ARO-RAGE, the company's investigational RNA interference (RNAi) therapeutic designed to reduce production of the receptor for advanced glycation end products (RAGE) as a potential treatment for asthma and other inflammatory pulmonary diseases.

James Hamilton, M.D., MBA, senior vice president of discovery and translational medicine at Arrowhead, said: "ARO-RAGE is a new clinical candidate using the pulmonary targeted, inhaled TRiM™ platform. We believe RAGE is a promising target as it represents an upstream mediator of the inflammatory cascade in asthma. Importantly, a soluble form of RAGE known as sRAGE can be assessed as a useful serum biomarker to measure gene target knockdown. We hope to further investigate the potential of ARO-RAGE and our pulmonary platform broadly in the upcoming clinical study, including assessments of safety, pharmacokinetics, and target engagement in a non-invasive way. These insights can potentially help to inform the development path of future pulmonary candidates."

An application for approval of the clinical trial was submitted to a local Ethics Committee and to the New Zealand Medicines and Medical Devices Safety Authority for review by the Standing Committee on Therapeutic Trials. Pending clearance, Arrowhead intends to proceed with ARORAGE-1001, a Phase 1/2a study to evaluate the safety, tolerability, pharmacokinetics, and pharmacodynamics of ARO-RAGE in up to 64 healthy volunteers and in up to 16 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.

Source: Arrowhead Pharmaceuticals, Inc.

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Source: Arrowhead Pharmaceuticals, Inc.