Arrowhead Pauses ARO-ENaC Phase 1/2 Clinical Study

July 2, 2021

PASADENA, Calif.--(BUSINESS WIRE)--Jul. 2, 2021-- Arrowhead Pharmaceuticals Inc. (NASDAQ: ARWR) today notified regulatory agencies, institutional review boards, and investigators that effective immediately it is voluntarily pausing AROENaC1001, a Phase 1/2 clinical study of ARO-ENaC, the company's investigational RNA interference (RNAi) therapeutic being developed as a treatment for patients with cystic fibrosis (CF), after receiving a preliminary update from an ongoing chronic toxicology study in rats that contained unexpected signals of local lung inflammation. The company has instructed investigators to pause new screening, enrollment, and any further dosing of investigational ARO-ENaC pending additional data from the ongoing chronic rat toxicology study and an additional ongoing chronic primate toxicology study.

Javier San Martin, M.D., chief medical officer at Arrowhead, said: "The safety of patients that participate in clinical trials of our investigational medicines is paramount to us at Arrowhead. While we have not seen any concerning safety or tolerability signals in subjects enrolled in the AROENaC1001 study, out of an abundance of caution we have decided to pause new screening, enrollment, and any further dosing of investigational ARO-ENaC in the study while we await additional information from ongoing nonclinical toxicology studies. After we receive the full data from these studies, we will assess whether there is an acceptable path forward for further clinical investigation. While we determine our next steps with ARO-ENaC, we look forward to continued progress with the other 8 clinical drug candidates in our pipeline as well as our rapidly expanding preclinical initiatives, including additional lung-targeted programs."

Christopher Anzalone, Ph.D., president and CEO at Arrowhead, said: "This is difficult news for cystic fibrosis patients who are in need of new therapeutic options, but we place above all else our obligation to ensure the safety of those enrolled in our clinical trials. Even though the preliminary information we received from the chronic toxicology study in rats may not necessarily bear directly upon the safety of continuing the current Phase 1 study, we believe that we need to better understand the findings and how they may translate to humans before we treat additional patients. This may delay our pulmonary program a bit, but it's just part of drug development. As the long-term toxicology data come in, we will work as quickly as we can to understand their implications for ARO-ENaC and the patients we hope to serve. We remain committed to the pulmonary platform and are moving ahead aggressively with our other pulmonary programs, two of which have already been nominated as clinical candidates."

AROENaC1001 (NCT04375514) is a Phase 1/2 dose-escalating study to evaluate the safety, tolerability, and pharmacokinetic effects of investigational ARO-ENaC in normal healthy volunteers and to evaluate the safety, tolerability, and efficacy in patients with CF.

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," "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, 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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