Arrowhead Pharmaceuticals Initiates Dosing Phase 1/2 Study of ARO-ENaC for Treatment of Cystic Fibrosis

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PASADENA, Calif.--(BUSINESS WIRE)--Aug. 11, 2020-- Arrowhead Pharmaceuticals Inc. (NASDAQ: ARWR) today announced that it has dosed the first subjects in 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), which is a rare disease caused by genetic mutations that lead to progressive deterioration in lung function due to poor clearance of mucus and associated recurrent infections. ARO-ENaC utilizes Arrowhead's proprietary Targeted RNAi Molecule (TRiM™) platform and is the company's first inhaled RNAi candidate to target pulmonary epithelium.

Javier San Martin, M.D., chief medical officer at Arrowhead, said: "The Phase 1/2 clinical study, AROENaC1001, is designed to assess safety, tolerability, and pharmacokinetics and potentially provide an early assessment of efficacy in patients with CF. We believe ENaC, or epithelial sodium channel, is a target with great potential for many CF patients that may not be eligible for existing therapies due to their specific genotypes, commonly called class I patients, and for those that have an inadequate response to therapy. Our preclinical work on ARO-ENaC has been highly promising and we are eager to see how these results translate to humans."

ARO-ENaC is designed to reduce activity of the epithelial sodium channel alpha subunit in the airways of the lung. In patients with CF, dysfunction in the cystic fibrosis transmembrane conductance regulator (CFTR) causes increased ENaC activity which contributes to airway dehydration and reduced mucociliary transport. This predisposes patients to persistent lung infections, structural damage, and progressive loss of pulmonary function. ENaC has been extensively explored as a potential therapeutic target for CF, but the development of inhaled small molecule ENaC inhibitors has been limited by on-target renal toxicity and short duration of action in the lung.

AROENaC1001 is a Phase 1/2 dose-escalating study to evaluate the safety, tolerability, and pharmacokinetic effects of ARO-ENaC in up to 24 normal healthy volunteers and to evaluate the safety, tolerability, and efficacy in up to 30 patients with CF. Exploratory objectives in patients with CF include assessing the effects of ARO-ENaC on changes in lung clearance index (LCI) and evaluating changes in forced expiratory volume (FEV1).

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. 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 various factors and uncertainties, including 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, and the enforcement of our intellectual property rights. Our most recent Annual Report on Form 10-K and subsequent Quarterly Reports on Form 10-Q discuss some of the important risk factors that may affect our business, results of operations and financial condition. We assume no obligation to update or revise forward-looking statements to reflect new events or circumstances.

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