Arrowhead Pharmaceuticals Presents Preclinical Data on ARO-ENaC at the North American Cystic Fibrosis Conference

October 31, 2019

PASADENA, Calif.--(BUSINESS WIRE)--Oct. 31, 2019-- Arrowhead Pharmaceuticals Inc. (NASDAQ: ARWR) today presented preclinical data at the 2019 North American Cystic Fibrosis Conference (NACFC) on ARO-ENaC, an inhaled RNAi therapeutic being developed as a potential treatment for cystic fibrosis (CF), which is a rare disease caused by genetic mutations that lead to mucus buildup in the lungs and pancreas. Arrowhead is currently conducting IND/CTA-enabling studies to support regulatory filings in the first half of 2020 for first-in-human studies.

Bruce Given, M.D., chief operating officer and head of R&D at Arrowhead, said: “We view the epithelial sodium channel, ENaC, as an exciting target in the treatment of CF and one that is well suited to an RNAi-based intervention. Based on its mechanism of action, ARO-ENaC has the potential to provide clinical benefit to all patients with CF, regardless of genotype, and may prove useful in combination with existing or new CFTR-targeted therapies. The data presented today show that ARO-ENaC can accelerate mucociliary clearance in normal sheep and also preserve airway physiology in a sheep disease model of impaired mucociliary clearance. In addition, animal models indicate that ARO-ENaC can reduce ENaC activity in the lung but avoid impact in the kidney, which has historically been a key safety liability for prior investigational small molecule ENaC inhibitors.”

ARO-ENaC is designed to reduce production of the epithelial sodium channel alpha subunit (αENaC) in the airways of the lung. In patients with CF, increased ENaC activity contributes to airway dehydration and reduced mucociliary transport. In CF lung disease, patients can have difficulty breathing and experience frequent and persistent lung infections. Various human genetic studies have validated ENaC 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.

Key points presented at NACFC 2019 include the following:

- Arrowhead's pulmonary epithelial TRiM ™-enabled delivery platform increased potency of RNAi trigger-mediated silencing of αENaC mRNA, and durably reduced whole lung gene expression in rodents
- In normal sheep, inhaled ARO-ENaC produced dose-dependent acceleration of mucociliary clearance, which is a lung physiology endpoint linked to pulmonary function in patients with CF
- Inhaled ARO-ENaC preserves lung clearance in a sheep disease model of mucostasis
- ARO-ENaC may offer a new renal-sparing, genotype-agnostic therapy for all patients with CF, and exhibiting the potential for an extended duration of action that should minimize treatment burden
- Arrowhead is also expanding its pulmonary delivery platform to address additional lung disease targets, particularly those that are inaccessible to traditional small molecule or antibody approaches

A copy of the poster presentation can be accessed on the Events and Presentations page under the Investors section of the Arrowhead website.

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.

Source: Arrowhead Pharmaceuticals, Inc.

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