October 23, 2017

Arrowhead Presents Promising Preclinical Data on Development of ARO-AAT for Treatment of Alpha-1 Liver Disease at Liver Meeting® 2017

Data Presented in Late Breaking Poster

Company Expects to File Clinical Trial Application in First Quarter 2018 to Begin First-in-Human Studies of ARO-AAT

PASADENA, Calif.--(BUSINESS WIRE)-- Arrowhead Pharmaceuticals Inc. (NASDAQ: ARWR) today presented data from studies demonstrating promising preclinical safety and efficacy of ARO-AAT, a second-generation investigational medicine for the treatment of alpha-1 liver disease that leverages Arrowhead's subcutaneously administered Targeted RNAi Molecule (TRiM™) technology. Based on these positive results, and pending completion of GLP toxicology studies, Arrowhead expects to file a clinical trial application in the first quarter of 2018 to begin first-in-human studies of ARO-AAT. The data were presented in a late breaking poster at the Liver Meeting® 2017, the Annual Meeting of the American Association for the Study of Liver Disease (AASLD), being held in Washington, D.C.

The poster presentation, titled, "Subcutaneous delivery of a RNA interference (RNAi) therapeutic candidate for alpha-1 antitrypsin deficiency (AATD)-related liver disease produces deep and prolonged knockdown of plasma AAT," highlighted the results of studies in which ARO-AAT produced deep and prolonged knockdown of alpha-1 antitrypsin (AAT) to levels that appear to be near full suppression of the liver production. The efficacy of ARO-AAT was evaluated in the transgenic PiZ mouse model and in nonhuman primates (NHPs) by measuring the reduction in plasma or serum levels of AAT. Inhibition of synthesis and subsequent reduction of the mutant Z-AAT accumulation in the livers of patients with alpha-1 liver disease may lead to the prevention, and potential reversal, of liver injury.

Preliminary safety of ARO-AAT was also assessed in rats and NHPs at dose levels up to and including 300 mg/kg, which is approximately 100 times the expected human clinical dose. In the rat study, clinical laboratory values were indistinguishable between groups receiving ARO-AAT and control groups. In addition, there were no histopathology findings deemed to be related to ARO-AAT. In the NHP study, there were no abnormal clinical observations, body weight changes, clinical chemistries, nor organ weight findings noted.

Poster Details:

Subcutaneous delivery of a RNA interference (RNAi) therapeutic candidate for alpha-1 antitrypsin deficiency (AATD)-related liver disease produces deep and prolonged knockdown of plasma AAT

- Publication Number: LB-21
- Session: Late-Breaking Poster Session
- Session Date and Time: October 23, 2017, from 8:00 AM to 5:30 PM
- Authors: Christine I. Wooddell, *et al.*

A copy of the poster may 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/alerts.cfm.

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 future success of our scientific studies, our ability to successfully develop 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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Arrowhead Pharmaceuticals, Inc.
Vince Anzalone, CFA
626-304-3400
ir@arrowheadpharma.com
or
Investors and Media:
LifeSci Advisors, LLC
Brian Ritchie
212-915-2578
britchie@lifesciadvisors.com
www.lifesciadvisors.com

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