October 20, 2016

Arrowhead Pharmaceuticals to Present Data on ARC-AAT at The Liver Meeting®

PASADENA, Calif.--(BUSINESS WIRE)-- Arrowhead Pharmaceuticals, Inc. (NASDAQ: ARWR) today announced that it will make two presentations on ARC-AAT, the company's investigational medicine for the treatment of liver disease associated with alpha-1 antitrypsin deficiency (AATD), at The Liver Meeting® 2016, the Annual Meeting of the American Association for the Study of Liver Disease (AASLD) being held on November 11-15, 2016, in Boston.

"At The Liver Meeting® we will present initial results from Part A of a Phase 1 study indicating that ARC-AAT is well-tolerated and induces deep, durable, and dose-dependent knockdown of circulating alpha-1 antitrypsin. In addition, we will present mouse model data showing that over time ARC-AAT had the desired effect of preventing liver production of mutant Z-alpha1 antitrypsin polymers and reversing liver disease," said Bruce Given, M.D., chief operating officer and head of R&D at Arrowhead. "The clinical abstract shows that ARC-AAT achieved reductions in serum AAT of up to 90%. Our previously reported primate studies indicate that approximately 10% of AAT may be produced outside the liver, so the Phase 1 clinical results suggest that we achieved near complete suppression of the liver produced AAT. This was an exciting result that gives us confidence as we conduct our ongoing Phase 2 multiple-dose study, which includes biopsies that will allow us to assess ARC-AAT's histological effects."

Oral Presentation:

RNA interference therapeutic ARC-AAT prevents production of Z-alpha1 antitrypsin polymers and reverses liver disease phenotype in PiZ mouse model

- Publication Number: 124
- Session: Parallel 19: Pediatric and Metabolic Liver Diseases: Basic and Translational
- Session Date and Time: November 13, 2016 from 4:45 PM to 6:15 PM
- Authors: Christine Wooddell, *et al.*

Poster Presentation:

RNA interference (RNAi) with ARC-AAT provides deep and prolonged knockdown of alpha-1 antitrypsin levels in healthy volunteers

- Publication Number: LB-24
- Session: Late-Breaking Poster Session
- Session Date and Time: November 13, 2016 from 8:00 AM to 5:30 PM
- Authors: Alice Turner, *et al.*

Additional details including presentation abstracts can be found on the AASLD website at http://www.aasld.org/. A copy of presentation materials can be accessed by visiting the Events section of the Arrowhead website after the presentations conclude.

About ARC-AAT

Arrowhead's ARC-AAT is being investigated for the treatment of liver disease associated with alpha-1 antitrypsin deficiency (AATD), a rare genetic disease that severely damages the liver and lungs of affected individuals. The mean estimated prevalence of AATD in the U.S. is 1 per 3000-5000, or approximately 100,000 patients. AATD is also an important cause of pediatric liver disease with an estimated prevalence in children of approximately 20,000 patients, and 50-80% likely to manifest liver disease during childhood. It is a rare disease that is frequently misdiagnosed or undiagnosed. ARC-AAT employs a novel unlocked nucleobase analog (UNA) containing RNAi trigger molecule designed for systemic delivery using the Dynamic Polyconjugate™ delivery system. ARC-AAT is highly effective at knocking down the alpha-1 antitrypsin (AAT) gene transcript and reducing the hepatic production of the mutant AAT (Z-AAT) protein in animal studies. Reduction of liver production of the inflammatory Z-AAT protein, which is believed to be the cause of progressive liver disease in AATD

patients, is important as it is expected to halt the progression of liver disease. ARC-AAT was granted orphan drug designation in both the United States and in Europe, the latter being held on Arrowhead's behalf by a local EU representative Pharma Gateway AB. Arrowhead is conducting a Phase 1 clinical study of ARC-AAT, with part A in healthy volunteers (now complete) and part B in AATD patients, and a Phase 2 multiple dose study in AATD patients.

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. The company's pipeline includes ARC-520 and ARC-521 for chronic hepatitis B virus infection, ARC-AAT for liver disease associated with alpha-1 antitrypsin deficiency, ARC-F12 for hereditary angioedema and thromboembolic disorders, ARC-LPA for cardiovascular disease, and ARC-HIF2 for renal cell carcinoma.

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 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.

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