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Arrowhead Pharmaceuticals Initiates Phase 2 Study of ARC-AAT

PASADENA, Calif.--(BUSINESS WIRE)-- Arrowhead Pharmaceuticals Inc. (NASDAQ: ARWR) today announced that it initiated a Phase 2 clinical study of ARC-AAT, an investigational RNAi-based medicine for the treatment of liver disease associated with alpha-1 antitrypsin deficiency (AATD). The study is designed to evaluate safety and tolerability and determine the effect of multiple doses of ARC-AAT on levels of circulating and intrahepatic alpha-1 antitrypsin as evidenced by changes in liver biopsy in patients with AATD.

Bruce Given, M.D., chief operating officer and head of R&D at Arrowhead said, "There remains no medical treatment for the liver disease associated with AATD, which is increasingly being recognized by patients and physicians as a serious problem. Our Phase 2 2001 study should give us, and the AATD community in general, the first insights into whether ARC-AAT can stop the progression of liver disease and possibly even allow the liver to recover and heal existing damage. This would be a significant breakthrough for patients."

The ARC-AAT Phase 2 study (ARCAAT2001) is a multi-center, open-label, multiple dose-escalation study of ARC-AAT in patients with AATD. In total, the study will consist of at least 8 and a maximum of 12 participants. Two dose levels, 4 mg/kg and 6 mg/kg, will be evaluated in two separate cohorts. All subjects will receive a pre-dose biopsy, seven doses of ARC-AAT (once every 28 days), and a post-dose biopsy completed at Day 183. The study will be conducted at multiple centers in Canada, Ireland, and Sweden. The Company may add additional centers in other countries, pending regulatory and ethics review.

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 models. 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 and potentially allow fibrotic tissue repair. 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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