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Arrowhead Initiates Dosing of ARC-AAT in Patients with Alpha-1 Antitrypsin Deficiency

PASADENA, Calif.--(BUSINESS WIRE)-- Arrowhead Research Corporation (NASDAQ: ARWR), a biopharmaceutical company developing targeted RNAi therapeutics, today announced that it dosed the first patient in Part B of a Phase 1 clinical trial of ARC-AAT. ARC-AAT is Arrowhead's RNAi-based drug candidate for the treatment of liver disease associated with the rare genetic disorder alpha-1 antitrypsin deficiency (AATD) that was recently granted orphan drug designation by the United States Food and Drug Administration. The clinical trial is currently enrolling patients at a single center in Australia and the company intends to open additional sites for enrollment in Europe, pending regulatory permission to proceed. The company expects to complete enrollment of the Phase 1 study by the end of 2015.

"Dosing the first alpha-1 patient with ARC-AAT is a milestone for Arrowhead and for patients with AATD. The goal of treatment with ARC-AAT is to halt progression and possibly reverse the liver injury and fibrosis associated with AATD, which currently has no approved therapy short of liver transplant. This is becoming a larger clinical problem that we believe ARC-AAT holds great potential to address," said Bruce D. Given, M.D., Arrowhead's Chief Operating Officer. "We would also like to thank the Alpha-1 Foundation and The Alpha-1 Project, who have agreed to help support the development of ARC-AAT through funding and assistance with patient recruitment."

The ongoing Phase 1 trial of ARC-AAT is a multi-center, randomized, placebo-controlled, double-blind, single dose-escalation, first-in-human study to evaluate the safety, tolerability and pharmacokinetics of ARC-AAT and the effect on circulating AAT levels. The study has been enrolling in dose cohorts of six participants each, with participants randomized at a ratio of 2:1 (active:placebo) to receive a single intravenous injection of either ARC-AAT or placebo (normal saline). The study consists of two parts; Part A in healthy volunteers, which has been completed, and Part B to be conducted in patients with PiZZ genotype AATD. Dosing in patients begins at the highest dose level used in healthy volunteers and then continued dose escalation may proceed under the protocol. The study evaluates participants for 28 days following dosing, with additional follow-up if needed every 2 weeks until AAT levels return to baseline.

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 appears to be frequently misdiagnosed or undiagnosed. ARC-AAT, which was granted orphan drug designation, 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 animals. 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. Arrowhead is conducting a single dose Phase 1 clinical study of ARC-AAT, with part A in healthy volunteers and part B in AATD patients.

About Arrowhead Research Corporation

Arrowhead Research Corporation is a biopharmaceutical company developing targeted RNAi therapeutics. The company is leveraging its proprietary Dynamic Polyconjugate™ delivery platform to develop targeted drugs based on the RNA interference mechanism that efficiently silences disease-causing genes. Arrowhead's pipeline includes ARC-520 for chronic hepatitis B virus and ARC-AAT for liver disease associated with Alpha-1 antitrypsin deficiency.

For more information please visit http://www.arrowheadresearch.com, or follow us on Twitter @ArrowRes. To be added to the Company's email list and receive news directly, please visit

http://ir.arrowheadresearch.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. Arrowhead Research Corporation's 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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