January 19, 2016

# Arrowhead's ARC-AAT Granted EMA Orphan Drug Designation

PASADENA, Calif.--(BUSINESS WIRE)-- Arrowhead Research Corporation (NASDAQ: ARWR), a biopharmaceutical company developing targeted RNAi therapeutics, today announced that the European Medicines Agency (EMA) granted Orphan Drug Designation to ARC-AAT for treatment of congenital alpha-1 antitrypsin deficiency. ARC-AAT was also previously granted Orphan Drug Designation by the United States Food and Drug Administration (FDA) in 2015.

Orphan Drug Designation from the EMA allows Arrowhead to benefit from a number of incentives, such as reduced regulatory fees, protocol assistance, and market exclusivity, to develop a medicine for the treatment of a rare disease affecting not more than five in 10,000 people in the European Union.

"ARC-AAT has the potential to provide patients and physicians with a much needed treatment option for liver disease associated with alpha-1 antitrypsin deficiency, a rare genetic disorder. We are pleased that the EMA has granted Orphan Drug Designation and we view this as an important regulatory milestone for the program," said Bruce Given, M.D., Arrowhead chief operating officer.

**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 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 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 and ARC-521 for chronic hepatitis B virus, ARC-AAT for liver disease associated with alpha-1 antitrypsin deficiency, ARC-F12 for hereditary angioedema and thromboembolic diseases, and ARC-HIF2 for renal cell carcinoma.

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, the timing of any market authorization, the timing of any market authorization for competitive products, 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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**Source:** Arrowhead Research Corporation

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