Arrowhead Begins Dosing in Phase 1 Study of ARO-AAT for Treatment of Alpha-1 Liver Disease

March 12, 2018

PASADENA, Calif.--(BUSINESS WIRE)--Mar. 12, 2018-- Arrowhead Pharmaceuticals Inc. (NASDAQ: ARWR) today announced that it has dosed the first subjects in a Phase 1 clinical study of ARO-AAT, which is being developed as treatment for a rare genetic liver disease associated with alpha-1 antitrypsin deficiency. ARO-AAT is the first clinical candidate to utilize Arrowhead's proprietary Targeted RNAi Molecule (TRiM^{TM}) technology. The second candidate, ARO-HBV, which is being developed as a potentially curative therapy for patients with chronic hepatitis B virus (HBV) infection, is also on schedule to dose the first subjects in a Phase 1/2 study at the end of March.

Bruce Given, M.D., chief operating officer and head of R&D at Arrowhead said, "There remains no adequate treatment for alpha-1 liver disease, other than transplant. ARO-AAT is designed to reduce the liver production of the inflammatory Z-AAT protein, which is believed to be the cause of progressive liver disease in AATD patients. This reduction may stop the progression of liver disease and possibly even allow the liver to recover and heal existing damage. This would represent a significant and much-needed breakthrough for patients."

The study, AROAAT1001 (NCT03362242), is a Phase 1 single- and multiple-ascending dose study to evaluate the safety, tolerability, pharmacokinetics, and effect of ARO-AAT on serum alpha-1 antitrypsin levels in healthy adult volunteers. The study is designed to include up to 5 cohorts of 8 subjects per cohort who will receive placebo or ARO-AAT at doses of 35, 100, 200, 300, or 400 mg.

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.

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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