Arrowhead Pharmaceuticals Files IND for Pivotal Phase 2/3 Study of ARO-AAT for Treatment of Alpha-1 Liver Disease

March 18, 2019

PASADENA, Calif.--(BUSINESS WIRE)--Mar. 18, 2019-- Arrowhead Pharmaceuticals Inc. (NASDAQ: ARWR) today announced that it has submitted an Investigational New Drug (IND) application to the U.S. Food and Drug Administration (FDA) for an adaptive Phase 2/3 trial with the potential to serve as a pivotal registrational study of ARO-AAT, the company's second generation subcutaneously administered RNA interference (RNAi) therapeutic being developed as a treatment for a rare genetic liver disease associated with alpha-1 antitrypsin deficiency (AATD).

Bruce Given, M.D., chief operating officer and head of R&D at Arrowhead, said: "Our discussions with the FDA have been productive and helpful as we moved through the process of determining the Phase 2/3 adaptive trial design with appropriate surrogate endpoints. We look forward to initiating this trial and moving closer to potentially providing patients, physicians, and the alpha-1 community with a much-needed therapy for alpha-1 liver disease."

Pending regulatory review, the company intends to initiate the adaptive design, Phase 2/3 study of ARO-AAT in patients with AATD associated liver disease. The proposed primary objectives are to evaluate safety and pharmacodynamic dose response, and to evaluate efficacy, defined as an improvement in a histologic grading scale of AATD associated liver disease, and no worsening of liver fibrosis based on Ishak score on end of study biopsy.

Arrowhead intends to initiate the study at various sites in the U.S. in the second quarter of 2019, followed by various international sites in Europe, pending regulatory submission and review. The company plans to provide additional study details following its initiation.

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/email-alerts.

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 likelihood and timing of the receipt of future milestone and licensing fees, the future success of our scientific studies, our ability to successfully develop and commercialize 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.

Source: Arrowhead Pharmaceuticals, Inc.

View source version on businesswire.com: https://www.businesswire.com/news/home/20190318005211/en/

Source: Arrowhead Pharmaceuticals, Inc.

Arrowhead Pharmaceuticals, Inc.
Vince Anzalone, CFA
626-304-3400
ir@arrowheadpharma.com

Investors and Media:
LifeSci Advisors, LLC
Brian Ritchie
212-915-2578
britchie@lifesciadvisors.com
www.lifesciadvisors.com