Arrowhead Pharmaceuticals Files for Regulatory Clearance to Begin Phase 1 Study of ARO-APOC3 for Treatment of Hypertriglyceridemia

January 7, 2019

PASADENA, Calif.--(BUSINESS WIRE)--Jan. 7, 2019-- Arrowhead Pharmaceuticals Inc. (NASDAQ: ARWR) today announced that it has filed an application for approval to begin a Phase 1 clinical trial of ARO-APOC3, an RNAi-based investigational medicine targeting Apolipoprotein C-III (apoC-III) being developed for the treatment of hypertriglyceridemia.

Chris Anzalone, Ph.D., president and CEO of Arrowhead Pharmaceuticals, said: “ARO-APOC3 will be our fifth product candidate leveraging the Targeted RNAi Molecule, or TRiM^{TM}, platform to enter clinical studies. Addressing hypertriglyceridemia through reduction of liver-produced apoC-III offers multiple potential development opportunities, including both orphan indications, such as FCS, and large market indications. In AROAPOC31001, our first-in-human study, we intend to evaluate ARO-APOC3 in healthy volunteers and various patient populations with elevated triglycerides, which will help inform our strategy around the ideal development and regulatory paths to pursue.”

Pending approval, Arrowhead intends to proceed with AROAPOC31001 (NCT03783377), a Phase 1 single and multiple dose-escalating study to evaluate the safety, tolerability, pharmacokinetics, and pharmacodynamic effects of ARO-APOC3 in adult healthy volunteers, hypertriglyceridemic patients, and patients with familial chylomicronemia syndrome (FCS). The study is designed to enroll up to 63 subjects.

The application for approval of the clinical trial is being submitted to a local Ethics Committee and to the New Zealand Medicines and Medical Devices Safety Authority for review by the Standing Committee on Therapeutic Trials.

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.

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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