Arrowhead Pharmaceuticals Files IND for Phase 2b Study of ARO-ANG3 for Treatment of Mixed Dyslipidemia

January 25, 2021

PASADENA, Calif.--(BUSINESS WIRE)--Jan. 25, 2021-- Arrowhead Pharmaceuticals Inc. (NASDAQ: ARWR) today announced that it has submitted an Investigational New Drug Application (IND) to the U.S. Food and Drug Administration (FDA) for a Phase 2b dose-finding clinical study of ARO-ANG3, the company's investigational RNA interference (RNAi) therapeutic being developed as a treatment for patients with mixed dyslipidemia.

Javier San Martin, M.D., chief medical officer at Arrowhead, said: "Cardiovascular disease remains the major cause of death, even after advances in therapies that reduce LDL cholesterol. This highlights the need for new therapies that can help patients at risk for cardiovascular disease to optimize their lipid profiles and protect them from atherosclerosis progression and cardiovascular events. By inhibiting *ANGPTL3*, Arrowhead's investigational ARO-ANG3 is designed to reduce triglycerides and decrease LDL cholesterol in patients with mixed dyslipidemia. We have been very encouraged by the clinical data from this program to date and look forward to further assessing the safety and efficacy of ARO-ANG3 in the upcoming Phase 2b study."

Following FDA's review of the IND, the company intends to initiate AROANG3-2001, a Phase 2b dose-finding clinical study in patients with elevated triglycerides and low-density lipoprotein cholesterol (LDL-C). The primary objective of the study is to evaluate the safety and efficacy of ARO-ANG3 in adults with mixed dyslipidemia and to select a dosing regimen for later stage clinical studies in this patient population. The study is designed to include a total of 180 participants in three cohorts. All dose cohorts will enroll in parallel, with 60 participants per cohort randomly assigned in a 3:1 ratio to receive ARO-ANG3 or placebo.

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.

View source version on businesswire.com: https://www.businesswire.com/news/home/20210125005267/en/

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

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

Media:
LifeSci Communications, LLC
Josephine Belluardo, Ph.D.
646-751-4361
jo@lifescicomms.com
www.lifescicommunications.com

Source: Arrowhead Pharmaceuticals, Inc.