Arrowhead Pharmaceuticals Files IND to Begin Phase 2b Study of ARO-APOC3 in Patients with Severe Hypertriglyceridemia

March 1, 2021

PASADENA, Calif.--(BUSINESS WIRE)--Mar. 1, 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 ARO-APOC3, the company's investigational RNA interference (RNAi) therapeutic being developed as a treatment for patients with hypertriglyceridemia.

Javier San Martin, M.D., chief medical officer at Arrowhead, said: "There is a clear need for new therapies that can reduce triglycerides across multiple patient populations, including those with the most severe elevations that can lead to recurrent bouts of pancreatitis. In the initial clinical study of ARO-APOC3, which we presented at the American Heart Association meeting last fall, triglycerides were lowered by up to 92% after just two doses. Based on these highly promising results, we intend to conduct Phase 2b and Phase 3 clinical studies across a wide spectrum of patient populations, pending review by regulators."

Following the FDA's review of the IND, the company intends to initiate a Phase 2b dose-finding clinical study (ARO-APOC3-2001) in patients with severe hypertriglyceridemia (sHTG). Additional clinical studies planned in 2021 include a Phase 2b dose-finding clinical study in patients with mixed dyslipidemia and a Phase 3 clinical study in patients with familial chylomicronemia syndrome (FCS).

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:
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

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