Arrowhead Pharmaceuticals Initiates Phase 2b Study of Investigational ARO-APOC3 for Treatment of Severe Hypertriglyceridemia

June 3, 2021

PASADENA, Calif.--(BUSINESS WIRE)--Jun. 3, 2021-- Arrowhead Pharmaceuticals Inc. (NASDAQ: ARWR) today announced that it has dosed the first patient in AROAPOC3-2001, a Phase 2b clinical study of ARO-APOC3, the company's investigational RNA interference (RNAi) therapeutic being developed as a treatment for patients with severe hypertriglyceridemia (SHTG). Arrowhead also intends to initiate a Phase 2b study and a Phase 3 study of ARO-APOC3 in two additional patient populations in 2021.

Javier San Martin, M.D., chief medical officer at Arrowhead, said: "There are approximately four million people in the United States alone with triglyceride levels greater than 500 mg/dL. Current treatments such as fibrates and fish oils generally seem to provide only modest reductions in triglycerides, and many people remain uncontrolled and at risk of developing pancreatitis. We believe there remains a significant need for new therapies that can substantially lower and sustain triglyceride levels in patients with severe hypertriglyceridemia. We reported initial results from a Phase 1 clinical trial of ARO-APOC3 at the American Heart Association Scientific Sessions in 2020. These results indicated that ARO-APOC3 reduced triglyceride levels by up to 92% with a long duration of effect that was maintained for longer than 12 weeks after the second dose. Importantly, ARO-APOC3 appeared to show consistent activity in various patient populations with a range of different baseline triglyceride levels. These encouraging results provide us with great confidence as we begin a Phase 2b clinical study, AROAPOC3-2001, in patients with severe hypertriglyceridemia to identify an optimal dose and regimen for later stage clinical trials."

AROAPOC3-2001 is a double-blind, placebo-controlled Phase 2b study to evaluate the efficacy and safety of ARO-APOC3 in adults with SHTG. Three dose levels of ARO-APOC3 (10 mg, 25 mg and 50 mg) will be evaluated against placebo in participants who have mean fasting triglycerides of greater than or equal to 500 mg/dL (5.65 mmol/L) at screening. A total of approximately 300 participants will be enrolled in the study. All dose cohorts will enroll in parallel with 100 participants per dose cohort randomly assigned in a 3:1 ratio to receive ARO-APOC3 or placebo. Each participant will receive subcutaneous injections on day 1 and week 12. The duration of the study is approximately 54 weeks from screening to the week 48 end-of-study examination. The primary objective of the study is to evaluate the safety and efficacy of ARO-APOC3 in adults with SHTG and to select a dosing regimen for later stage clinical studies in this patient population.

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. Any statements contained in this release except for historical information may be deemed to be forward-looking statements. Without limiting the generality of the foregoing, words such as "may," "will," "expect," "believe," "anticipate," "intend," "plan," "project," "could," "estimate," or "continue" are intended to identify such forward-looking statements. In addition, any statements that refer to projections of our future financial performance, trends in our businesses, or other characterizations of future events or circumstances are forward-looking statements. 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 numerous factors and uncertainties, including the continuing impact of the COVID-19 pandemic, 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, the enforcement of our intellectual property rights, and the other risks and uncertainties described in our most recent Annual Report on Form 10-K, subsequent Quarterly Reports on Form 10-Q and other documents filed with the Securities and Exchange Commission from time to time. We assume no obligation to update or revise forward-looking statements to reflect new events or circumstances.

Source: Arrowhead Pharmaceuticals, Inc.

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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

Source: Arrowhead Pharmaceuticals Inc.