# Arrowhead Pharmaceuticals Initiates Phase 2b Study of ARO-APOC3 for Treatment of Mixed Dyslipidemia

September 30, 2021

PASADENA, Calif.--(BUSINESS WIRE)--Sep. 30, 2021-- Arrowhead Pharmaceuticals, Inc. (NASDAQ: ARWR) today announced that it has dosed the first patient in AROAPOC3-2002, a Phase 2b clinical study of ARO-APOC3 in adults with mixed dyslipidemia. ARO-APOC3 is the company's investigational RNA interference (RNAi) therapeutic being developed as a treatment for patients with severe hypertriglyceridemia (SHTG), mixed dyslipidemia, and familial chylomicronemia syndrome (FCS). Arrowhead intends to initiate a Phase 3 study of ARO-APOC3 in patients with FCS in the fourth quarter of 2021.

Javier San Martin, M.D., chief medical officer at Arrowhead, said: "AROAPOC3-2002 is the second of three clinical trials that we plan to initiate this year to study investigational ARO-APOC3 in various patient populations, each with a unique lipid profile. The '2001 study, which we initiated in June of 2021, is enrolling patients with severe hypertriglyceridemia. The '2002 study is enrolling patients with mixed dyslipidemia, defined by moderately elevated triglycerides and elevated non-high-density lipoprotein cholesterol or elevated low-density lipoprotein cholesterol at screening. The planned '3001 study, which is on track to begin in the fourth quarter of 2021, will be a Phase 3 study in patients with genetically defined FCS. We look forward to continued progress for the clinical program and, if successful, providing new potential treatment options for patients with various levels of hypertriglyceridemia."

AROAPOC3-2002 (NCT04998201) is a double-blind, placebo-controlled Phase 2b study to evaluate the efficacy and safety of ARO-APOC3 in adults with mixed dyslipidemia. Four dose cohorts of ARO-APOC3 will be evaluated against placebo in participants who had the following at screening: (i) elevated triglycerides (TG) greater than or equal to 150 mg/dL (1.69 mmol/L) but less than 499 mg/dL (5.61 mmol/L), and (ii) non-high-density lipoprotein cholesterol (non-HDL-C) greater than or equal to 100 mg/dL (2.59 mmol/L) or low-density lipoprotein cholesterol (LDL-C) greater than or equal to 70 mg/dL (1.8 mmol/L) at screening. A total of approximately 320 participants will be enrolled in the study. All dose cohorts will enroll in parallel with approximately 80 participants per dose cohort, randomly assigned in a 3:1 ratio to receive ARO-APOC3 or placebo. In three cohorts (10 mg, 25 mg, 50 mg), each participant will receive a subcutaneous injection on day 1 and week 12 for a total of 2 injections. In one additional 50 mg cohort, each participant will receive a subcutaneous injection on day 1 and week 24 for a total of 2 injections. 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 mixed dyslipidemia and to select a dose and 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.*

www.lifescicommunications.com

**Source:** Arrowhead Pharmaceuticals, Inc.

View source version on businesswire.com: https://www.businesswire.com/news/home/20210930005132/en/

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

Source: Arrowhead Pharmaceuticals, Inc.