Arrowhead Pharmaceuticals Initiates Phase 2b Study of Investigational ARO-ANG3 for Treatment of Mixed Dyslipidemia

June 30, 2021

PASADENA, Calif.--(BUSINESS WIRE)--Jun. 30, 2021-- Arrowhead Pharmaceuticals Inc. (NASDAQ: ARWR) today announced that it has dosed the first patient in AROANG3-2001, a Phase 2b 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: "By inhibiting *ANGPTL3*, Arrowhead's investigational ARO-ANG3 is designed to reduce triglycerides and LDL cholesterol in patients with mixed dyslipidemia. Patients with persistently elevated triglycerides and LDL cholesterol may have a higher risk of atherosclerosis progression and cardiovascular events, which are major causes of death. The Phase 2b AROANG3-2001 clinical study will evaluate safety and efficacy, and identify an optimal dose and dosing regimen of ARO-ANG3 to be assessed in future late-stage clinical studies."

AROANG3-2001 (NCT04832971) is a double-blind, placebo-controlled Phase 2b study to evaluate the efficacy and safety of investigational ARO-ANG3 in adults with mixed dyslipidemia. Three dose levels of ARO-ANG3 (50 mg, 100 mg, and 200 mg) will be evaluated against placebo in participants with mixed dyslipidemia who had the following at screening: (i) low-density lipoprotein cholesterol (LDL-C) greater than or equal to 70 mg/dL (1.8 mmol/L) or non-high-density lipoprotein cholesterol (non-HDL-C) greater than or equal to 100 mg/dL (2.59 mmol/L) and (ii) mean fasting triglycerides (TG) greater than or equal to 150 mg/dL (1.69 mmol/L) but less than 499 mg/dL (5.61 mmol/L). A total of approximately 180 participants will be enrolled in the study. All dose cohorts will enroll in parallel with 60 participants per cohort randomly assigned in a 3:1 ratio to receive a subcutaneous injection of ARO-ANG3 or placebo on day 1 and week 12. The duration of the study is approximately 42 weeks from screening to the week 36 end-of-study examination. After completing the week 36 visit, participants will be eligible to continue in an open-label extension study. The primary objective of the AROANG3-2001 study is to evaluate the safety and efficacy of ARO-ANG3 in adults with mixed dyslipidemia and 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 business, expectations for our product pipeline or product candidates, including anticipated regulatory submissions and clinical program results, prospects or benefits of our collaborations with other companies, 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 impact of the ongoing COVID-19 pandemic on our business, 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.

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