# Arrowhead Pharmaceuticals Initiates Phase 3 PALISADE Study of ARO-APOC3 for Treatment of Familial Chylomicronemia Syndrome

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PASADENA, Calif.--(BUSINESS WIRE)--Jan. 12, 2022-- Arrowhead Pharmaceuticals Inc. (NASDAQ: ARWR) today announced that it has dosed the first patients in the PALISADE study, a Phase 3 clinical study to evaluate the efficacy and safety of ARO-APOC3 in adults with familial chylomicronemia syndrome (FCS). ARO-APOC3 is the company's investigational RNA interference (RNAi) therapeutic that is designed to inhibit the production of apolipoprotein C3 (APOC3), a key regulator of triglyceride metabolism. ARO-APOC3 is currently being investigated in multiple clinical studies, including the Phase 3 PALISADE study in patients with FCS, the Phase 2b SHASTA-2 study in patients with severe hypertriglyceridemia (SHTG), and the Phase 2b MUIR study in patients with mixed dyslipidemia.

Javier San Martin, M.D., chief medical officer at Arrowhead, said: "Initiating the Phase 3 PALISADE study is an important milestone for the ARO-APOC3 program, for patients with FCS, and for Arrowhead. Patients with genetically defined FCS, and those who are phenotypically similar, tend to have extremely high triglycerides, which can lead to an elevated risk of recurrent and painful bouts of pancreatitis. These patients currently have very limited treatment options. In the previously conducted Phase 1/2 clinical study of ARO-APOC3 in 4 genetically confirmed FCS patients, ARO-APOC3 treatment led to a maximal mean reduction in APOC3 of up to 98%, a maximal median decrease in triglycerides of up to 91% and was generally well-tolerated. These changes are similar to those observed in 26 participants that had SHTG and similar baseline triglyceride levels but did not carry biallelic mutations consistent with FCS. These results were presented in November 2021 at the American Heart Association Scientific Sessions meeting[1]. We look forward to investigating ARO-APOC3 in the larger Phase 3 PALISADE study to further assess its efficacy and safety."

Arrowhead is partnering with Ambry Genetics to provide no-cost genetic testing to patients who may be at risk of severe hypertriglyceridemia and FCS. To learn more about this program, visit the Ambry program site, here.

PALISADE (NCT05089084) is a global, placebo-controlled Phase 3 study to evaluate the efficacy and safety of ARO-APOC3 in adult participants with FCS. Approximately 60 participants who have met all protocol eligibility criteria during screening will be randomized in a double-blinded fashion to receive ARO-APOC3, or matching placebo, administered subcutaneously once every 3 months. Participants will be randomly assigned 2:1:2:1 to the dose cohorts (ARO-APOC3 25 mg, volume-matched placebo, ARO-APOC3 50 mg, and volume-matched placebo, respectively). The study will enroll participants with fasting triglycerides greater than or equal to 10 mmol/L (greater than or equal to 880 mg/dL) that are refractory to standard lipid-lowering therapy and a diagnosis of FCS as defined in the inclusion criteria. The duration of the study is approximately 56 weeks from screening to the month 12 end-of-study examination. After month 12, participants will be eligible to continue in an open-label extension study. All participants in the placebo group who opt to continue will switch to active drug during the extension study. The primary objective of the study is to evaluate the change from baseline in triglycerides between each ARO-APOC3 dose and pooled placebo at month 10.

**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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[1] Peter Clifton et al., Presented by Christie Ballantyne on behalf of Peter Clifton, AHA Scientific Sessions 2021

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