Arrowhead Pharmaceuticals Completes Enrollment of Phase 3 PALISADE Clinical Trial Evaluating ARO-APOC3 for Treatment of Familial Chylomicronemia Syndrome

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- Primary Study Completion Anticipated in the Second Quarter of 2024

PASADENA, Calif.--(BUSINESS WIRE)--May 16, 2023-- Arrowhead Pharmaceuticals Inc. (NASDAQ: ARWR) today announced that it has completed enrollment of its global Phase 3 clinical trial (PALISADE) evaluating ARO-APOC3 for the treatment of familial chylomicronemia syndrome. The company anticipates that the primary portion of the study will be complete in the second quarter of 2024 with a data readout shortly thereafter and subsequent submissions for regulatory review and approval.

Javier San Martin, M.D., chief medical officer at Arrowhead, said: “Completing enrollment of the Phase 3 PALISADE study represents a significant milestone for Arrowhead and brings our potentially important RNAi-based medicines closer to the patients that may benefit from them. I am grateful to the FCS patient community and principal investigators for their trust and participation in the PALISADE study.”

About the Familial Chylomicronemia Syndrome

Familial chylomicronemia syndrome (FCS) is a severe and ultrarare genetic disease often caused by various monogenic mutations. FCS leads to extremely high triglyceride (TG) levels, typically over 900 mg/dL. Such severe elevations can lead to various serious signs and symptoms including acute and potentially fatal pancreatitis, chronic abdominal pain, type 2 diabetes mellitus, hepatic steatosis, and cognitive issues. Currently, the therapeutic options that can adequately treat FCS are very limited.

About the PALISADE Phase 3 Study

The AROAPOC3-3001 PALISADE study (NCT05089084) is a Phase 3 placebo controlled study to evaluate the efficacy and safety of ARO-APOC3 in adults with FCS. The primary endpoint of the study is percent change from baseline in fasting TG at Month 10. A total of 75 subjects distributed across 39 different sites in 18 countries were randomized to receive 25 mg ARO-APOC3, 50 mg ARO-APOC3, or matching placebo once every three months. Participants who complete the randomized period are eligible to continue in a 2-part extension period, where all participants will receive ARO-APOC3.

About ARO-APOC3

ARO-APOC3 is an investigational RNAi therapeutic being developed as a treatment for patients with mixed dyslipidemia, severe hypertriglyceridemia, and FCS. ARO-APOC3 is designed to reduce production of Apolipoprotein C-III (apoC-III), a component of triglyceride rich lipoproteins (TRLs) including very-low-density lipoprotein (VLDL) and chylomicrons and is a key regulator of triglyceride metabolism. Knocking down the hepatic production of apoC-III by RNAi may result in reduced VLDL synthesis and assembly, enhanced breakdown of TRLs, and better clearance of VLDL and chylomicron remnants.

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,” “hope,” “intend,” “plan,” “project,” “could,” “estimate,” “continue,” “target,” “forecast” or “continue” or the negative of these words or other variations thereof or comparable terminology 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 forward-looking statements include, but are not limited to, statements about the initiation, timing, progress and results of our preclinical studies and clinical trials, and our research and development programs; our expectations regarding the potential benefits of the partnership, licensing and/or collaboration arrangements and other strategic arrangements and transactions we have entered into or may enter into in the future; our beliefs and expectations regarding milestone, royalty or other payments that could be due to or from third parties under existing agreements; and our estimates regarding future revenues, research and development expenses, capital requirements and payments to third parties. 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, decisions of regulatory authorities and the timing thereof, 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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