Arrowhead Pharmaceuticals Initiates Phase 2 GATEWAY Study of Investigational ARO-ANG3 for Treatment of Homozygous Familial Hypercholesterolemia

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PASADENA, Calif.--(BUSINESS WIRE)--Apr. 26, 2022-- Arrowhead Pharmaceuticals Inc. (NASDAQ: ARWR) today announced that it has dosed the first patients in the Phase 2 GATEWAY clinical study of investigational ARO-ANG3 for the treatment of patients with homozygous familial hypercholesterolemia (HoFH). ARO-ANG3 is the company's investigational RNA interference (RNAi) therapeutic designed to silence the hepatic expression of angiopoietin-like protein 3 (ANGPTL3), a liver synthesized inhibitor of lipoprotein lipase and endothelial lipase, being developed as a treatment for patients with mixed dyslipidemia.

Javier San Martin, M.D., chief medical officer at Arrowhead, said: "HoFH is the most serious and rare form of familial hypercholesterolemia. If untreated, HoFH can cause heart disease beginning in the early teen years. The GATEWAY Phase 2 clinical study will evaluate efficacy and safety in patients with HoFH, so we can assess the potential of ARO-ANG3 to be a new treatment option for patients who still have significant unmet medical need."

GATEWAY, also called AROANG3-2003 (NCT05217667), is an open-label Phase 2 clinical study to evaluate the efficacy and safety of investigational ARO-ANG3 in up to 16 subjects with HoFH. Two dose levels of ARO-ANG3 (200 mg and 300 mg) will be evaluated in subjects with documented HoFH based on genotype or clinical criteria, and with fasting LDL-C greater 100 mg/dL (2.59 mmol/L) and fasting triglycerides less than 300 mg/dL (3.39 mmol/L) at screening. Subjects will receive a subcutaneous injection of ARO-ANG3 on day 1 and day 84 and may be eligible to participate in an optional open-label extension study. The primary objective of the GATEWAY study is to evaluate the efficacy and safety of ARO-ANG3 in subjects with HoFH and the primary endpoint is the percent change in fasting calculated LDL-C from baseline to week 24.

Arrowhead recently completed enrollment in the ongoing Phase 2b ARCHES-2 clinical study evaluating ARO-ANG3 in more than 180 patients with elevated low-density lipoprotein LDL-C and triglycerides. The completion of this study is anticipated around the end of this year and the company intends to release topline data in the first half of 2023.

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," 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, 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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