Arrowhead Pharmaceuticals Receives Orphan Drug Designation for ARO-APOC3

June 21, 2019

PASADENA, Calif.--(BUSINESS WIRE)--Jun. 21, 2019-- Arrowhead Pharmaceuticals, Inc. (NASDAQ: ARWR) today announced that the U.S. Food and Drug Administration (FDA) has granted orphan drug designation to ARO-APOC3 for the treatment of familial chylomicronemia syndrome (FCS). There is no currently available therapy in the U.S. that can adequately treat FCS.

ARO-APOC3 is a subcutaneously administered RNAi therapeutic targeting apolipoprotein C-III (APOC3) currently being developed as a potential treatment for patients with severe hypertriglyceridemia and FCS. The company began dosing ARO-APOC3 in a first-in-human study in March 2019. The trial is a Phase 1 single and multiple dose study to evaluate the safety, tolerability, pharmacokinetics, and pharmacodynamic effects of ARO-APOC3 in up to 63 adult healthy volunteers with elevated triglycerides and patients with severe hypertriglyceridemia and FCS.

The mission of the FDA's Office of Orphan Products Development (OOPD) is to advance the evaluation and development of products that demonstrate promise for the diagnosis and/or treatment of rare diseases or conditions that affect fewer than 200,000 people in the U.S. In fulfilling that task, the OOPD evaluates scientific and clinical data submissions from sponsors to identify and designate products as promising for rare diseases and to further advance scientific development of such promising medical products. Orphan drug designation provides incentives for sponsors to develop products for rare diseases. These incentives may include a partial tax credit for certain clinical trial expenditures, the waiver of certain FDA user fees, and potential eligibility for seven years of orphan drug marketing exclusivity, if approved.

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. 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 various factors and uncertainties, including 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, and the enforcement of our intellectual property rights. Our most recent Annual Report on Form 10-K and subsequent Quarterly Reports on Form 10-Q discuss some of the important risk factors that may affect our business, results of operations and financial condition. We assume no obligation to update or revise forward-looking statements to reflect new events or circumstances.

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