Arrowhead Pharmaceuticals Doses First Patient in SEQUOIA Phase 2/3 Study of ARO-AAT for Treatment of Alpha-1 Liver Disease

August 8, 2019

PASADENA, Calif.--(BUSINESS WIRE)--Aug. 8, 2019-- Arrowhead Pharmaceuticals Inc. (NASDAQ: ARWR) today announced that it has dosed the first patient in SEQUOIA (AROAAT2001), a potentially pivotal Phase 2/3 clinical study of ARO-AAT, the company's second generation subcutaneously administered RNA interference (RNAi) therapeutic being developed as a treatment for a rare genetic liver disease associated with alpha-1 antitrypsin deficiency (AATD).

Bruce Given, M.D., chief operating officer and head of R&D at Arrowhead, said: "ARO-AAT is the first RNAi therapeutic derived from our proprietary Targeted RNAi Molecule, or TRiM™, platform to reach a potentially pivotal study. This is a significant milestone for Arrowhead and, more importantly, it represents potential hope for patients living with alpha-1 liver disease, who currently have no available treatment options other than liver transplant."

SEQUOIA (NCT03945292) is a placebo-controlled, adaptive design Phase 2/3 study to evaluate the safety, efficacy, and tolerability of ARO-AAT administered subcutaneously to patients with AATD associated liver disease. SEQUOIA is designed to enroll 120 patients who will receive at least 9 doses, or approximately two years of treatment, with ARO-AAT or placebo. Doses will be administered on day 1, 29, and approximately every 12 weeks thereafter. The four-arm placebo-controlled Part A component of the study will feed seamlessly into a two-arm placebo-controlled Part B component. The primary objective for Part A is to select a single dose level for use in Part B of the study based on a combined evaluation of safety and pharmacodynamic dose response in each Part A cohort using change from baseline in soluble liver mutant AAT (Z-AAT), insoluble liver Z-AAT, and serum AAT levels as pharmacodynamic metrics. The primary objective for Part B is to evaluate efficacy, as assessed by the proportion of ARO-AAT treated patients relative to placebo achieving a 2-point improvement in a histologic grading scale of AATD associated liver disease, and no worsening of liver fibrosis on end of study biopsy.

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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