Arrowhead Pharmaceuticals Initiates Phase 1/2 Study of ARO-C3 for Treatment of Complement Mediated Diseases

February 18, 2022

PASADENA, Calif.--(BUSINESS WIRE)--Feb. 18, 2022-- Arrowhead Pharmaceuticals Inc. (NASDAQ: ARWR) today announced that it has dosed the first subjects in AROC3-1001, a Phase 1/2 clinical study of ARO-C3, the company's investigational RNA interference (RNAi) therapeutic designed to reduce production of complement component 3 (C3) as a potential therapy for various complement mediated diseases.

James Hamilton, M.D., MBA, senior vice president of discovery and translational medicine at Arrowhead, said: "We believe a C3 targeted drug has the potential to address multiple complement mediated and complement associated diseases, where significant unmet medical need exists. These include IgA nephropathy, C3 glomerulopathy, paroxysmal nocturnal hemoglobinuria, and additional renal and hematologic diseases that we intend to evaluate in the future. ARO-C3 has progressed rapidly, and our preclinical data have been very encouraging. We are eager to continue this progress as we evaluate ARO-C3 in clinical studies."

AROC3-1001 (NCT05083364) is a Phase 1/2, placebo controlled, dose-escalating study to evaluate the safety, tolerability, pharmacokinetics, and pharmacodynamics of ARO-C3 in up to 24 adult healthy volunteers, up to 24 adult patients with paroxysmal nocturnal hemoglobinuria (PNH), and up to 14 adult patients with complement-mediated renal disease. In Part 1 in healthy volunteers, four cohorts with four escalating dose levels of ARO-C3 will be evaluated. Each cohort will enroll 6 eligible subjects who will be randomized to receive a single subcutaneous injection of ARO-C3 (n=4) or placebo (n=2). In Part 2, eligible subjects with PNH or complement-mediated renal disease will be enrolled to receive open-label ARO-C3 on day 1 and day 85 at one of two dose levels to be determined in Part 1. The primary objective of the study is to evaluate the safety, tolerability, pharmacokinetics, and pharmacodynamics of single doses of ARO-C3 in normal healthy volunteers and to evaluate the safety, tolerability, pharmacokinetics, and pharmacodynamics of multiple doses of ARO-C3 in subjects with PNH and in subjects with complement-mediated renal disease.

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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