Arrowhead Files for Regulatory Clearance to Begin Phase 1/2a Study of ARO-C3 for Treatment of Complement Mediated Diseases

October 25, 2021

- KOL Webinar Tomorrow, October 26, 2021 at 3:00 p.m. ET

PASADENA, Calif.--(BUSINESS WIRE)--Oct. 25, 2021-- Arrowhead Pharmaceuticals Inc. (NASDAQ: ARWR) today announced that it has filed an application for clearance to begin a Phase 1/2a clinical trial of ARO-C3, the company's investigational RNA interference (RNAi) therapeutic being developed as a treatment for patients with various complement mediated diseases. Arrowhead will host a key opinion leader (KOL) webinar on October 26, 2021, at 3:00 p.m. ET to discuss ARO-C3 and the company's development plans.

Chris Anzalone, Ph.D., president and chief executive officer of Arrowhead, said: "ARO-C3 utilizes Arrowhead's proprietary TRiM™ platform to silence the hepatic production of complement C3. This will be our tenth TRiM™-enabled clinical program and the eighth targeting a liver expressed protein. In all of our liver-targeted programs to date, we have seen consistent and efficient translation of preclinical *in vivo* results to human clinical results, so we have a high degree of confidence in investigational ARO-C3. In addition, we view ARO-C3 as having the potential to address multiple serious complement-mediated or complement-associated diseases with unmet need in the renal and hematology space. We look forward to discussing the program in more detail on October 26, 2021 when we host a webinar, during which key opinion leaders will describe some of the diseases that may potentially be addressed by ARO-C3. These include IgA nephropathy, C3 glomerulopathy, and paroxysmal nocturnal hemoglobinuria."

An application for approval of the clinical trial is being submitted to a local Ethics Committee and to the New Zealand Medicines and Medical Devices Safety Authority for review by the Standing Committee on Therapeutic Trials. Pending clearance, Arrowhead intends to proceed with AROC3-1001, a Phase 1/2a dose-escalating study to evaluate the safety, tolerability, pharmacokinetics, and pharmacodynamics of ARO-C3 in up to 24 adult healthy volunteers and in up to 24 adult patients with paroxysmal nocturnal hemoglobinuria and up to 14 adult patients with complement-mediated renal disease.

The webinar will feature presentations by KOLs Richard J. Glassock, MD, MACP, FRCP, FASN (David Geffen School of Medicine at UCLA), and Peter Browett, BMedSci, MBChB, FRACP, FRCPA (University of Auckland School of Medicine). They will discuss the current treatment landscape and unmet medical need in treating patients with complement mediated renal and hematological diseases. Arrowhead's management team will discuss the biology of the target, preclinical data generated to date, and clinical plans for ARO-C3. ARO-C3 is designed to reduce production of complement component 3 (C3) as a potential therapy for various complement mediated diseases.

The webinar may be accessed on the Events and Presentations page under the Investors section of the Arrowhead website on October 26, and a replay will be made available following the conclusion of the webinar.

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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