Arrowhead Announces Interim Results from Ongoing Phase 1/2 Study of ARO-C3 for Treatment of Complement Mediated Diseases

February 28, 2023

- Achieved Mean Reductions of 88% in C3 and 91% in AH50

PASADENA, Calif.--(BUSINESS WIRE)--Feb. 28, 2023-- Arrowhead Pharmaceuticals Inc. (NASDAQ: ARWR) today announced interim results from Part 1 of AROC3-1001, an ongoing 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. The company plans to present additional results at an upcoming complement-focused medical meeting. Dosing in Part 2 of the Phase 1/2 study is expected to begin in the first half of 2023.

In Part 1 of AROC3-1001, ARO-C3 interim results included:

- A dose-dependent reduction in serum C3, with 88% mean reduction at highest dose tested
- A dose-dependent reduction in AH50, a marker of alternative complement pathway hemolytic activity, with 91% mean reduction at highest dose tested
- Duration of pharmacologic effect supportive of quarterly or less frequent subcutaneous dose administration
- Safety and tolerability
    - Overall, no clinically significant laboratory findings or patterns of adverse changes in any clinical laboratory parameters
    - No dose limiting toxicity, serious or severe adverse events, or study discontinuation due to adverse events
    - Most common adverse events include headache, COVID-19, generally mild injection site reactions, and seasonal allergy

"ARO-C3 has achieved encouraging results in Part 1 of this Phase 1/2 clinical study, including a mean reduction of 88% in C3 and 91% in AH50 at the highest dose tested. These data in healthy volunteers provide us with further confidence as we begin Part 2 of the study, which includes patients with various complement mediated diseases," said James Hamilton, M.D., MBA, chief of discovery and translational medicine at Arrowhead. "Substantial unmet medical need remains in the treatment of multiple complement mediated diseases, including IgA nephropathy, C3 glomerulopathy, paroxysmal nocturnal hemoglobinuria, and additional renal and hematologic indications, despite the availability of approved complement C5 inhibitors that have significantly improved treatment. In addition, we believe C3 inhibition has interesting potential, as it is upstream of C5 in the complement cascade."

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 42 adult healthy volunteers (Part 1), and up to 42 adult patients with paroxysmal nocturnal hemoglobinuria (PNH) or with complement-mediated renal disease (Part 2).

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," "continue," "target," "forecast" or "continue" or the negative of these words or other variations thereof or comparable terminology 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 forward-looking statements include, but are not limited to, statements about the initiation, timing, progress and results of our preclinical studies and clinical trials, and our research and development programs; our expectations regarding the potential benefits of the partnership, licensing and/or collaboration arrangements and other strategic arrangements and transactions we have entered into or may enter into in the future; our beliefs and expectations regarding milestone, royalty or other payments that could be due to or from third parties under existing agreements; and our estimates regarding future revenues, research and development expenses, capital requirements and payments to third parties. 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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