Arrowhead Pharmaceuticals Files for Regulatory Clearance to Initiate Phase 1/2a Study of ARO-CFB for Complement Mediated Kidney Disease

December 21, 2023

PASADENA, Calif.--(BUSINESS WIRE)--Dec. 21, 2023-- Arrowhead Pharmaceuticals Inc. (NASDAQ: ARWR) today announced that it has filed an application for clearance to initiate a Phase 1/2a clinical trial of ARO-CFB, the company's investigational RNA interference (RNAi) therapeutic being developed as a potential treatment for complement mediated renal disease, such as immunoglobulin A nephropathy (IgAN), which is the most common glomerular disease worldwide and carries a high lifetime risk of progression to end-stage renal disease. Additionally, ARO-CFB may have clinical applications in non-renal diseases involving complement activation.

ARO-CFB is designed to reduce hepatic expression of complement factor B (CFB), which plays an important regulatory role in amplifying complement alternative pathway activation and has been identified as a promising therapeutic target.

James Hamilton, M.D., MBA, Chief of Discovery and Translational Medicine at Arrowhead, said: "IgAN is the most common glomerular disease worldwide, accounting for up to 40% of all cases of glomerulonephritis. Our preclinical studies have demonstrated that ARO-CFB can achieve deep and durable reductions in liver production of CFB, which plays a key role in the activation of the alternative complement pathway associated with the pathogenesis of renal disease in IgAN. As the liver is the main site of CFB production and our TRiM^{TM} system has consistently demonstrated the ability to silence liver production of target gene products across several candidates, we believe ARO-CFB has the potential to be a powerful treatment option for complement mediated renal disease."

An application for approval of the clinical trial was 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 AROCFB-1001, a Phase 1/2a dose-escalating study to evaluate the safety, tolerability, pharmacokinetics, and pharmacodynamics of ARO-CFB in up to 66 healthy volunteers and patients with complement mediated kidney 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 X (formerly Twitter) at @ArrowheadPharma or on LinkedIn. 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.

Source: Arrowhead Pharmaceuticals, Inc.

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Source: Arrowhead Pharmaceuticals, Inc.