Arrowhead Pharmaceuticals Files IND for Phase 1b Study of ARO-HIF2 for Treatment of Clear Cell Renal Cell Carcinoma

December 11, 2019

PASADENA, Calif.--(BUSINESS WIRE)--Dec. 11, 2019-- Arrowhead Pharmaceuticals Inc. (NASDAQ: ARWR) today announced that it has submitted an Investigational New Drug (IND) application to the U.S. Food and Drug Administration (FDA) for a Phase 1b adaptive dose-finding clinical study of ARO-HIF2, the company's investigational RNA interference (RNAi) therapeutic being developed as a treatment for patients with clear cell renal cell carcinoma (ccRCC).

Javier San Martin, M.D., chief medical officer at Arrowhead, said: "ARO-HIF2 is Arrowhead's first TRiM™ enabled investigational RNAi therapeutic to target a cell type outside of the liver. Clear cell renal cell carcinoma, or ccRCC, is one of the most common forms of kidney cancer. Most patients with ccRCC have a mutation in the Von Hippel-Lindau gene, rendering them unable to degrade HIF-2α, which leads to accumulation during tumor hypoxia and promotes tumor growth. We believe this makes HIF-2α an attractive target for an RNAi-based intervention."

Pending regulatory review, the company intends to initiate AROHIF21001 (NCT04169711), a Phase 1b adaptive design dose-finding clinical study in patients with advanced ccRCC to evaluate the safety of ARO-HIF2 and to determine the recommended Phase 2 dose. Additional secondary objectives include the assessment of pharmacokinetics and efficacy, based on Response Evaluation Criteria in Solid Tumors (RECIST). An exploratory objective for AROHIF21001 will be gene target knockdown using tumor 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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