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

August 18, 2020

PASADENA, Calif.--(BUSINESS WIRE)--Aug. 18, 2020-- Arrowhead Pharmaceuticals Inc. (NASDAQ: ARWR) today announced that it has dosed the first patient in AROHIF21001, a Phase 1b 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: "This Phase 1b study is designed to provide us with a robust readout on safety and dose response of ARO-HIF2, and may also deliver signals on preliminary efficacy in an advanced ccRCC patient population. Most patients with ccRCC, one of the most common forms of kidney cancer, have an inactivated mutation in the Von Hippel-Lindau gene rendering them unable to degrade HIF-2α, which leads to an increase in activity during tumor hypoxia and promotes tumor growth. We believe that HIF-2α is an attractive target for RNAi-based gene silencing and, more broadly, we are eager to achieve clinical validation for our tumor targeted TRiM ™-enabled RNAi therapeutics so we can address additional unmet needs in oncology. We also recently announced that ARO-ENaC, our investigational RNAi therapeutic targeting cystic fibrosis, started dosing in a Phase 1/2 study. This means that our TRiM ™ platform is now being studied in three different tissue types in human clinical trials. This is very exciting for us at Arrowhead and, we think, represents a significant step towards reaching the full therapeutic potential of RNA interference."

AROHIF21001 (NCT04169711) is a Phase 1b dose-finding clinical study in up to 18 patients with advanced ccRCC to evaluate the safety of ARO-HIF2 and to determine the recommended Phase 2 dose. Secondary objectives include the assessment of pharmacokinetics and preliminary efficacy, based on Response Evaluation Criteria in Solid Tumors (RECIST). Exploratory objectives for AROHIF21001 are post-dose tumoral expression of HIF genes in response to treatment with ARO-HIF2, change in Karnofsky Performance Status (KPS), correlation of tumor response based on RECIST with tumor HIF2α gene expression and tumor integrin expression, correlation of integrin expression with changes in HIF gene expression, evaluation of serum biomarkers of ARO-HIF2 activity, correlation of RCC-related gene expression to ARO-HIF2 activity, and evaluation of plasma and urine metabolites.

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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Arrowhead Pharmaceuticals, Inc.
Vince Anzalone, CFA
626-304-3400
ir@arrowheadpharma.com

Investors:
LifeSci Advisors, LLC
Brian Ritchie
212-915-2578
britchie@lifesciadvisors.com
www.lifesciadvisors.com

Media:
LifeSci Communications, LLC
Josephine Belluardo, Ph.D.
646-751-4361
jo@lifescicomms.com
www.lifescicommunications.com

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