Arrowhead Pharmaceuticals Initiates Phase 1/2 Study of ARO-HSD in Normal Healthy Volunteers and Patients with NASH or Suspected NASH

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PASADENA, Calif.--(BUSINESS WIRE)-- Arrowhead Pharmaceuticals Inc. (NASDAQ: ARWR) today announced that it has dosed the first patients in AROHSD1001, a Phase 1/2 clinical study of ARO-HSD, the company's investigational RNA interference (RNAi) therapeutic being developed as a treatment for patients with alcohol related and nonalcohol related liver diseases, such as nonalcoholic steatohepatitis (NASH).

Javier San Martin, M.D., chief medical officer at Arrowhead, said: "Genetic studies have recently pointed to HSD17B13 as a compelling target for multiple forms of liver disease. No other therapeutics against this target have reached clinical studies, so ARO-HSD is the first investigational medicine using any modality to initiate a clinical program to study whether HSD17B13 inhibition in the liver leads to a clinical benefit for patients. Further, based on our preclinical work and key findings from various studies with our other product candidates, we believe that an RNAi-based intervention that utilizes our proprietary TRiM^{TM} platform has the potential to be highly active at silencing the expression of HSD17B13."

AROHSD1001 (NCT04202354) is a Phase 1/2 single and multiple dose-escalating study to evaluate the safety, tolerability, pharmacokinetics, and pharmacodynamic effects of ARO-HSD in up to 74 normal healthy volunteers and patients with NASH or suspected NASH. Additional exploratory objectives include the assessment of various measures of drug activity using liver biopsy.

HSD17B13 is a member of the hydroxysteroid dehydrogenase family involved in the metabolism of hormones, fatty acids, and bile acids. Published human genetic data indicate that a loss of function mutation in HSD17B13 provides strong protection against alcoholic hepatitis, cirrhosis, and NASH, with approximately 30-50% risk reduction compared to non-carriers.[1]

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.

Source: Arrowhead Pharmaceuticals, Inc.

[1] The New England Journal of Medicine. 2018, 1096-1106

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