Arrowhead Presents Additional Clinical Data on Investigational ARO-HSD Treatment at AASLD Liver Meeting

November 12, 2021

PASADENA, Calif.--(BUSINESS WIRE)--Nov. 12, 2021-- Arrowhead Pharmaceuticals Inc. (NASDAQ: ARWR) today announced additional interim clinical data from AROHSD1001, an ongoing 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). The data were presented in a late-breaking poster at The Liver Meeting, the Annual Meeting of the American Association for the Study of Liver Disease (AASLD), taking place November 12-15, 2021.

Key data presented include the following:

Pharmacodynamic Response

- Dose-dependent pharmacodynamic effect on hepatic HSD17B13 mRNA was observed in all patients
  - At 200 mg, all patients showed greater than 90% mRNA reductions
- Hepatic HSD17B13 protein levels were reduced at all ARO-HSD dose levels, with multiple measurements below the assay's lower limit of quantitation
- Decreases in ALT and AST were observed at doses of 100 mg ARO-HSD and greater
- 9 of 18 patients had liver fat reductions on MRI-PDFF of 4-41%
- 6 of 18 patients had reduction in liver stiffness (kPa) on FibroScan of 4-37%
- Pharmacodynamic effect was not impacted by HSD17B13 (rs72613567, T>TA) or PNPLA3 (rs738409, C>G) mutations

Safety

- ARO-HSD was well-tolerated in patients, with no ARO-HSD-related serious adverse events reported, no adverse events leading to drug discontinuations, and no ARO-HSD-related clinically significant adverse laboratory trends observed
- One treatment emergent serious adverse event of soft tissue injury that required hospitalization was reported in cohort 4b. This event was considered unrelated to study drug

A copy of the poster may be accessed on the Events and Presentations page under the Investors section of the Arrowhead website.

ARO-HSD is an investigational RNAi therapeutic targeting HSD17B13 as a potential treatment for patients with alcohol-related and nonalcohol related liver diseases, such as NASH. 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] ARO-HSD is being investigated in AROHSD1001 (NCT04202354), 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 of AROHSD1001 include the assessment of various measures of drug activity using liver 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 may contain 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 news 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," "intend," "plan," "project," "could," "estimate," or "continue" 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. The forward-looking statements in this news release speak only as of the original date of this news release. 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, 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 contained in this news release to reflect new events or circumstances.

Source: Arrowhead Pharmaceuticals, Inc.

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

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