Arrowhead Pharmaceuticals Enters Exclusive License Agreement with GSK for ARO-HSD

November 22, 2021

- Upon closing, Arrowhead to receive $120 million upfront payment for Phase 1/2 program for NASH

- Arrowhead eligible to receive potential milestone payments and royalties on commercial sales

- GSK to receive an exclusive license for ARO-HSD in all territories except Greater China

PASADENA, Calif.--(BUSINESS WIRE)--Nov. 22, 2021-- Arrowhead Pharmaceuticals Inc. (NASDAQ: ARWR) today announced that it entered into an exclusive license agreement with GlaxoSmithKline (LSE/NYSE: GSK) under which GSK will develop and commercialize ARO-HSD, Arrowhead's investigational RNA interference (RNAi) therapeutic in a Phase 1/2 trial that is currently being developed as a treatment for patients with nonalcoholic steatohepatitis (NASH).

"Genetic studies have shown that HSD17B13 is a compelling therapeutic target for multiple forms of liver disease. Based on the clinical results generated to date, including those recently presented at the 2021 AASLD Liver Meeting, ARO-HSD could have the potential to be the first investigational therapeutic to achieve robust reductions in mRNA and protein levels of hepatic HSD17B13, leading to reductions in ALT, a liver enzyme typically elevated in liver diseases such as NASH," said Christopher Anzalone, Ph.D., Arrowhead's president and CEO. "GSK has a global reach and impressive capabilities in the clinical development and commercialization of important medicines. As the work continues towards progressing further Phase 2 studies and planning Phase 3 studies for ARO-HSD, we believe this agreement with GSK furthers the potential of ARO-HSD as a promising new investigational therapeutic for patients with NASH and other liver diseases. We look forward to enabling GSK to advance ARO-HSD towards the millions of patients worldwide who do not have adequate treatment options."

"NASH can be a life-threatening disorder in which excessive fat build-up in the liver causes inflammatory damage and fibrosis. The compelling genetic evidence linking HSD17B13 variants with protection of the liver from inflammatory injury suggests that there is an opportunity to produce a first-in-class medicine to reduce the clinical consequences of NASH. It's exciting to advance Arrowhead's promising RNAi approach into Phase 2 studies as an important potential new medicine for patients with NASH," said John Lepore, SVP and Head of Research, GSK.

Financial Terms

Under the terms of the agreement, Arrowhead will receive an upfront payment of $120 million and is eligible for additional payments of $30 million at the start of Phase 2 and $100 million upon achieving a successful Phase 2 trial readout and the first patient dosed in a Phase 3 trial. Furthermore, should the Phase 3 trial read out positively, and the potential new medicine receives regulatory approval in major markets, the deal provides for commercial milestone payments to Arrowhead of up to $190 million at first commercial sale, and up to $590 million in sales-related milestone payments. Arrowhead is further eligible to receive tiered royalties on net product sales.

GSK will receive an exclusive license to develop and commercialize ARO-HSD in all territories except Greater China, which will be retained by Arrowhead. GSK will be wholly responsible for further clinical development and commercialization, outside of Greater China.

The transaction is expected to close in the first quarter of 2022, subject to customary closing conditions and clearance under the Hart-Scott-Rodino Antitrust Improvements Act of 1976, as amended.

About ARO-HSD

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 trial 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.

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

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