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

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PASADENA, Calif.--(BUSINESS WIRE)--Nov. 12, 2021-- Arrowhead Pharmaceuticals Inc. (NASDAQ: ARWR) today announced additional interim clinical data from the ongoing AROAAT2002 study, an open-label Phase 2 clinical study of ARO-AAT, also known as TAK-999, the company's second-generation investigational RNA interference (RNAi) therapeutic being co-developed with Takeda Pharmaceutical Company Limited ("Takeda") as a treatment for the rare genetic liver disease associated with alpha-1 antitrypsin deficiency (AATD). 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

- ARO-AAT treatment allowed clearance of liver Z-AAT protein
  - Total liver Z-AAT was reduced by 72-100%
- ARO-AAT treatment improved liver fibrosis
  - 6 of 14 patients had a 1 point or greater improvement in METAVIR fibrosis stage from baseline to week 24 or 48
  - 6 of 14 patients had no change from baseline to week 24 or 48
  - 2 of 14 patients had an increase from F2 at baseline to F3 at week 48, although both patients had profound reduction in PAS+D globule burden and reduced ALT and GGT after treatment
- ARO-AAT treatment reduced histological globule burden
  - 13 of 13 patients had a 1 point or greater reduction in PAS+D globule burden
- ARO-AAT treatment improved biomarkers of liver health
  - Mean reduction from baseline ranged from 42% to 56% for ALT and from 33% to 54% for GGT at week 28 and week 72
  - All groups showed normalized ALT and GGT following treatment

Safety

- No treatment emergent adverse events leading to drug discontinuation, dose interruptions, or study withdrawal
- No treatment emergent adverse events related to change in pulmonary status or pulmonary function were reported
- No clinically meaningful changes in ppFEV1 from baseline (mean 85% [N=16]) were observed at Week 40 (mean 81% [N=15]) or at Week 72 (mean 84% [N=4])
- Four SAEs were reported: EBV-related myocarditis, diverticulitis, dyspnea, and vestibular neuronitis, all of which involve confounding factors or alternative etiology

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

In October 2020, Arrowhead and Takeda Pharmaceutical Company Limited announced a collaboration and licensing agreement to develop ARO-AAT. Under the terms of the agreement, Arrowhead and Takeda will co-develop ARO-AAT which, if approved, will be co-commercialized in the U.S. under a 50/50 profit-sharing structure. Outside the U.S., Takeda will lead the global commercialization strategy and receive an exclusive license to commercialize ARO-AAT with Arrowhead eligible to receive tiered royalties of 20-25% on net sales. Arrowhead will receive an upfront payment of $300 million and is eligible to receive potential development, regulatory and commercial milestones up to $740 million.

AROAAT2002 (NCT03946449) is a pilot open-label, multi-dose, Phase 2 study to assess the response to ARO-AAT in approximately 16 patients with AATD associated liver disease and baseline liver fibrosis who will be enrolled in three cohorts. All eligible participants will require a pre-dose biopsy and an end of study biopsy. Treated participants will also be offered the opportunity to continue treatment in an open-label extension (OLE). Including the OLE, interim assessments will be made after 6 months and 18 months (cohorts 1, 1b), and 12 months and 24 months (cohort 2) of treatment with ARO-AAT. Arrowhead is also evaluating ARO-AAT in the ongoing SEQUOIA study.

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.

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