



April 24, 2017

Arrowhead Pharmaceuticals Presents ARC-AAT Clinical Data at The International Liver Congress™

PASADENA, Calif.--(BUSINESS WIRE)-- Arrowhead Pharmaceuticals Inc. (NASDAQ: ARWR) presented clinical data from a Phase 1a/1b study of ARC-AAT, the company's prior generation investigational medicine that was being studied for the treatment of liver disease associated with alpha-1 antitrypsin deficiency (AATD), at The International Liver Congress™ 2017 (ILC), the annual meeting of the European Association for the Study of the Liver (EASL).

The data demonstrated that an RNA interference (RNAi) therapeutic can achieve deep durable, and dose dependent reductions of alpha-1 antitrypsin (AAT), with the highest dose studied in healthy volunteers achieving near full suppression of the liver production of the AAT protein. In addition, at doses of 2 and 4 mg/kg in AATD patients, ARC-AAT produced similar levels of knockdown as seen in healthy volunteers. These data support the continued advancement of ARO-AAT, Arrowhead's follow-on product candidate that utilizes the company's next generation, proprietary subcutaneously administered delivery vehicle.

Bruce Given, M.D., chief operating officer and head of R&D for Arrowhead Pharmaceuticals, said: "The data presented showed strong congruence of activity in primate studies, in normal healthy volunteers, and in patients with AATD. We believe these results, together with those from preclinical studies presented at AASLD last fall that showed that treatment with ARC-AAT over time may improve liver health and prevent further damage, provide proof-of-concept for the use of an RNAi therapeutic against alpha-1 liver disease."

Presentation materials can be accessed by visiting the [Events](#) section of the Arrowhead website. Additional details including abstracts can be found on the ILC website at <https://ilc-congress.eu/> by searching for the following:

Oral Presentation:

Hepatic targeted RNA interference provides deep and prolonged knockdown of alpha-1 antitrypsin levels in ZZ patients

- | Presentation Reference: LBO-06
- | Session: Late Breaker session
- | Date and Time: April 22, 2017 at 5:15 PM CET
- | Authors: Alice Turner, *et al.*

Arrowhead announced on November 29, 2016, that it had discontinued development of ARC-AAT. The company is currently focused on developing ARO-AAT, Arrowhead's follow-on investigational RNAi therapeutic against alpha-1 liver disease.

The International Liver Congress is a trademark of the European Association for the Study of the Liver.

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](https://twitter.com/ArrowheadPharma). To be added to the Company's email list and receive news directly, please visit <http://ir.arrowheadpharma.com/alerts.cfm>.

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 future success of our scientific studies, our ability to successfully develop 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.

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