April 20, 2017

Arrowhead Presents ARC-520 and ARC-521 Clinical Data at The International Liver Congress™

PASADENA, Calif.--(BUSINESS WIRE)-- Arrowhead Pharmaceuticals Inc. (NASDAQ: ARWR) today presented clinical data from a Phase 2 study of ARC-520 and a Phase 1/2 study of ARC-521, the company's prior generation investigational medicines that were being studied for the treatment of chronic hepatitis B virus (HBV) infection, at The International Liver Congress™ 2017 (ILC), the annual meeting of the European Association for the Study of the Liver (EASL).

Together, these presentations demonstrate that in human clinical studies, RNAi interference (RNAi) as a mechanism can rapidly and significantly reduce HBV viral antigens. In addition, RNAi appears to synergize with current standard-of-care nucleotide and nucleoside analogues (NUCs) to rapidly lower serum levels of HBV DNA. Arrowhead is currently developing ARO-HBV, a follow-on product candidate that utilizes the company's next generation, proprietary subcutaneously administered delivery vehicle, as a potentially curative therapy for patients with chronic hepatitis B infection.

Bruce Given, M.D., chief operating officer, head of R&D for Arrowhead Pharmaceuticals, said: "Arrowhead's ARC-520 and ARC-521 clinical studies have generated important data that continue to inform and guide the development path of ARO-HBV, our follow-on candidate against chronic HBV. We saw reductions in hepatitis B surface antigen, or HBsAg, of up to 3 logs following multiple doses with ARC-520 in combination with a NUC. HBV DNA was reduced by several logs and became undetectable in many patients. In addition, early data from the ARC-521 study indicate that RNAi therapeutics can be active against HBV gene products derived from both cccDNA and integrated HBV DNA. These data are consistent with findings from a long-term study in chimpanzees that we reported on previously. We view these results as validation for the use of RNAi in HBV, and believe ARO-HBV has the potential to serve as a cornerstone therapy for combinations intended to cure chronic HBV."

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:

Prolonged RNA interference therapy with ARC-520 Injection in treatment naïve, HBeAg positive and negative patients with chronic HBV results in significant reductions of HBs antigen

- Presentation Reference: PS-045
- Session: Parallel session: Hepatitis B and D: Emerging treatment options
- Date and Time: April 20, 2017 at 5:30 PM CET
- Authors: Man-Fung Yuen, *et al.*

Poster Presentation:

A phase 1 study to evaluate safety and tolerability of escalating single doses of the HBV RNA interference drug ARC-521 in a healthy volunteer population

- Presentation Reference: THU-176
- Session: Viral hepatitis: Hepatitis B and D - Clinical (therapy, new compounds, resistance)
- Session Date and Time: April 20, 2017 from 8:00 AM to 6:00 PM CET
- Authors: Edward Gane, *et al.*

Arrowhead announced on November 29, 2016 that it had discontinued development of ARC-520, ARC-521. The company continues to develop ARO-HBV, which is a follow on investigational RNAi therapeutic against chronic HBV that utilizes the company's next generation, proprietary subcutaneously administered delivery vehicle.

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. 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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