November 29, 2016

Arrowhead Pharmaceuticals Focuses Resources on Subcutaneous and Extra-Hepatic RNAi Therapeutics

- Conference Call and Webcast Today at 4:30 p.m. EST

PASADENA, Calif.--(BUSINESS WIRE)-- Arrowhead Pharmaceuticals, Inc. (NASDAQ: ARWR) today announced a strategic redeployment of resources to support the development of RNAi therapeutics that utilize the company's new proprietary subcutaneous (subQ) and extra-hepatic delivery systems. Arrowhead will discontinue development of clinical stage drug candidates ARC-520, ARC-521, and ARC-AAT, which utilize the DPC_{iv}™, or EX1, delivery vehicle. The company is hosting a conference call at 4:30 p.m. EST to discuss this decision.

Arrowhead remains committed to finding therapeutic options for patients with chronic hepatitis B (HBV) infection and alpha-1 antitrypsin deficiency (AATD). The company intends to advance to the clinic two previously unannounced HBV and AATD programs using our subQ platform. Arrowhead has learned a great deal during prior HBV and AAT studies that will help drive the subQ programs efficiently.

Existing preclinical subQ and extra-hepatic programs such as ARC-LPA and ARC-AMG1, which are partnered with Amgen, ARC-F12, ARC-HIF2, and other unannounced programs are not affected by this decision.

Because of the discontinuation of its existing clinical programs, the company is reducing its workforce by approximately 30%, while maintaining full resourcing necessary to support current and potential future partner-based programs and Arrowhead's burgeoning pipeline. This more streamlined structure should enable the company to continue to develop its programs rapidly, and is intended to extend its cash runway into 2019.

The decision to discontinue development of EX1-containing programs was based primarily on two factors. First, during ongoing discussions with regulatory agencies and outside experts, it became apparent that there would be substantial delays in all clinical programs that utilize EX1, while the company further explored the cause of deaths in a non-clinical toxicology study in non-human primates. Second, Arrowhead has made substantial advances in RNA chemistry and targeting resulting in large potency gains for subQ administered and extra-hepatic RNAi-based development programs. In preclinical studies with the subQ platform, the company has obtained depth and duration of target gene knockdown approaching that of intravenously administered EX1-containing candidates, at lower doses and with good safety margins.

The company believes it is prudent to focus its development resources entirely on its subQ and extra-hepatic pipeline, which includes programs in HBV, AAT, Factor 12, HIF-2alpha, and other unannounced programs. In addition to its own pipeline, Arrowhead is also focused on providing full resources to support its partnership with Amgen and potential future partnerships for its subQ and extra-hepatic delivery systems.

The tolerability of ARC-520, ARC-521, and ARC-AAT in human clinical trials appeared to be favorable, and in the company's view, supported advancing the programs. EX1-containing candidates have been administered over 800 times in more than 300 human study subjects and patients and have been generally well tolerated, with a small minority (6%) of infusions being associated with infusion reactions. In addition, across the ARC-520, ARC-521, and ARC-AAT clinical programs, laboratory values have not been deemed indicative of drug induced organ toxicity.

In addition, each candidate was highly active against its respective target. For example, data presented earlier this month at The Liver Meeting® show that ARC-AAT achieved 90% knockdown of serum AAT, which is believed to be near full suppression of liver production of the protein, in a Phase 1 clinical study. For ARC-520, it was previously reported that reductions in surface antigen (HBsAg) of almost 99%, or 2 logs, were achieved after a single dose. In subsequent multiple dose studies, for which data have not yet been reported, reductions of almost 3 logs were observed, with several patients being tracked that appear poised to possibly seroclear HBsAg, representing potential function cures.

However, due to likely regulatory considerations, as of this announcement all patient recruitment for ARC-520, ARC-521, and ARC-AAT has been halted and dosing discontinued. The company will work together with investigators and clinical sites to ensure a smooth transition of study closure and patient medical care.

Conference Call and Webcast Details

Investors may access a live audio webcast on the Company's website at http://ir.arrowheadpharma.com/events.cfm. For analysts that wish to participate in the conference call, please dial 855-215-6159 or 315-625-6887 and enter Conference ID 27048601.

A replay of the webcast will be available on the company's website approximately two hours after the conclusion of the call and will remain available for 90 days. An audio replay will also be available approximately two hours after the conclusion of the call and will be available for 3 days. To access the audio replay, dial 404-537-3406 and enter Conference ID 27048601.

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.

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