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Arrowhead Pharmaceuticals Doses First Patient with Hepatitis B in Multiple Ascending Dose Portion of Phase 1/2 Study of ARC-521

PASADENA, Calif.--(BUSINESS WIRE)-- Arrowhead Pharmaceuticals Inc. (NASDAQ: ARWR) today announced that it has dosed the first patient in the multiple ascending dose (MAD) portion of its ongoing Phase 1/2 study of ARC-521, the company's second RNAi-based investigational medicine for the treatment of chronic hepatitis B virus (HBV) infection. To date 24 healthy volunteers have been treated in the study, and the drug safety committee (DSC) approved initiation of the MAD after a planned review of safety data from cohort 3 of the healthy volunteer portion of the study. The MAD is designed to evaluate the safety, tolerability, and antiviral activity of single and multiple doses of ARC-521 in patients with chronic HBV.

Bruce Given, M.D., chief operating officer and head of R&D at Arrowhead said, "The innovative design for the first-in-man study of ARC-521 is intended to get to multiple dose data in patients rapidly. Arrowhead's development staff and the experienced investigators working on the study are operating at best-in-class speeds, enabling us to begin the MAD portion of the study just three months into the clinical program. This study may have multiple readouts, including single dose safety data in healthy volunteers and single and multiple dose safety and antiviral activity data in HBV patients. These readouts should happen progressively, potentially starting during the first quarter of 2017."

The MAD portion of the ARC-521 study (ARC5211001) is a multicenter, sequential, multiple dose, open label study in patients with chronic HBV. Up to 24 chronic HBV patients (8 patients per dose level) that are negative for Hepatitis B e-antigen (HBeAg) at screening will enroll sequentially into up to 3 dose levels (2 mg/kg, 4 mg/kg, 6 mg/kg) to receive 3 monthly doses of open label ARC-521.

About ARC-521

Arrowhead's ARC-521 is being investigated for its potential to produce functional cures in patients with chronic hepatitis B virus (HBV) infection. ARC-521 intervenes upstream of the reverse transcription process where current standard-of-care nucleotide and nucleoside analogs act, and is designed to silence the production of all HBV gene products. The small interfering RNAs (siRNAs) in ARC-521 engage the body's normal cellular RNAi machinery and direct specific cleavage of HBV RNA transcripts, thereby reducing the levels of HBV proteins and the RNA template used to produce viral DNA. Designed to complement ARC-520, ARC-521 is a second generation HBV candidate that targets HBV mRNA transcripts from both cccDNA and integrated DNA and is expected to be most suitable for those patients who tend to have lower levels of viral cccDNA. Arrowhead is investigating ARC-521 specifically to determine if significantly reducing circulating and non-circulating viral proteins and RNA will allow for re-constitution of an effective host immune response and ultimately HBsAg seroclearance resulting in functional cure. As many as 350-400 million people worldwide are chronically infected with the hepatitis B virus, which can lead to cirrhosis of the liver and is responsible for 80% of primary liver cancers globally. Arrowhead is conducting a Phase 1/2 single and multiple dose study in healthy volunteers and HBV patients.

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. The company's pipeline includes ARC-520 and ARC-521 for chronic hepatitis B virus infection, ARC-AAT for liver disease associated with alpha-1 antitrypsin deficiency, ARC-F12 for hereditary angioedema and thromboembolic disorders, ARC-LPA for cardiovascular disease, and ARC-HIF2 for renal cell carcinoma.

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 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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