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Arrowhead Pharmaceuticals Files for Regulatory Clearance to Begin Phase 1/2 Study of ARC-521

PASADENA, Calif.--(BUSINESS WIRE)-- Arrowhead Pharmaceuticals, Inc. (NASDAQ: ARWR), recently filed a regulatory submission in New Zealand to begin a Phase 1/2 clinical trial of ARC-521, its RNAi-based therapeutic candidate for the treatment of chronic hepatitis B virus (HBV) infection. Pending approval, Arrowhead intends to proceed with ARC521-1001, a first-in-human study to assess single and multiple-doses of ARC-521 in healthy volunteers and HBV patients.

Chris Anzalone, Ph.D., president and CEO of Arrowhead Pharmaceuticals, said: "ARC-521 is our second pipeline product targeting chronic HBV and was designed to silence gene products from both HBV cccDNA and integrated HBV DNA. This is important because our clinical work with ARC-520 in HBV patients and our long-term chimpanzee study suggest that different patient groups can have different relative levels of cccDNA. We think having both ARC-520, which has been very active in patients with higher cccDNA, and ARC-521, which may be optimal for those with lower cccDNA, should provide us with the potential to treat all patients with HBV. We have an aggressive plan for the development of ARC-521 that includes an accelerated first-in-man Phase 1/2 design intended to allow rapid transition into multi-dose patient cohorts."

The application for approval of a clinical trial was submitted to the New Zealand Medicines and Medical Devices Safety Authority (MEDSAFE) for review by the Standing Committee on Therapeutic Trials (SCOTT). Arrowhead also intends to seek regulatory clearance to conduct ARC521-1001 in additional countries.

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 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 planning to conduct 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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