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Arrowhead Files IND for RNAi Therapeutic ARC-520 to Begin Phase 2b Multiple-Dose Studies in Chronic Hepatitis B Patients

PASADENA, Calif.--(BUSINESS WIRE)-- Arrowhead Research Corporation (NASDAQ: ARWR), a biopharmaceutical company developing targeted RNAi therapeutics, today announced that it submitted an Investigational New Drug (IND) application to the U.S. Food and Drug Administration for ARC-520, it's drug candidate for the treatment of chronic hepatitis B virus (HBV) infection. Arrowhead plans to submit additional clinical trial authorization applications with regulatory authorities in various jurisdictions in Europe and Asia.

Pending national regulatory review and approval, Arrowhead intends to initiate two Phase 2b, multicenter, randomized, double-blind, placebo-controlled, multi-dose studies to determine the depth of hepatitis B surface antigen (HBsAg) reduction following ARC-520 injection in combination with entecavir or tenofovir in patients with chronic immune active HBV infection. The Heparc-2002 study is planned to include patients who are negative for hepatitis B e-antigen (HBeAg) at screening and the Heparc-2003 study is planned to include patients who are positive for HBeAg at screening. The primary objective is to evaluate the depth of HBsAg decline in response to multiple doses of ARC-520 compared to placebo in patients with chronic HBV infection as a measure of drug activity. Secondary objectives are to assess measures of safety and tolerability and to evaluate multi-dose pharmacokinetics of ARC-520 in patients with chronic HBV infection when co-administered with a fixed dose of entecavir or tenofovir, in addition to other exploratory safety and pharmacodynamic objectives.

"As with our recent application to begin a Phase 1 study with ARC-AAT, this IND represents achievement of a key 2014 goal," said Arrowhead President and CEO, Dr. Christopher Anzalone. "We look forward to beginning multi-dose studies with ARC-520."

About ARC-520

Arrowhead's RNAi-based candidate ARC-520 is being investigated in the treatment of chronic HBV infection. The small interfering RNAs (siRNAs) in ARC-520 intervene at the mRNA level, upstream of the reverse transcription process where current standard of care nucleotide and nucleoside analogues act. Arrowhead is investigating ARC-520 specifically, to determine if it can be used to achieve a functional cure, which is an immune clearant state characterized by hepatitis B s-antigen negative serum with or without sero-conversion. Arrowhead has completed a Phase 1 single ascending dose study in normal volunteers and the company is conducting a single dose Phase 2a study in chronic HBV patients. Approximately 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.

About Arrowhead Research Corporation

Arrowhead Research Corporation is a biopharmaceutical company developing targeted RNAi therapeutics. The company is leveraging its proprietary Dynamic Polyconjugate™ delivery platform to develop targeted drugs based on the RNA interference mechanism that efficiently silences disease-causing genes. Arrowhead's pipeline includes ARC-520 for chronic hepatitis B virus, ARC-AAT for liver disease associated with Alpha-1 antitrypsin deficiency, and partner-based programs in obesity and oncology.

For more information please visit http://www.arrowheadresearch.com, or follow us on Twitter @ArrowRes. To be added to the Company's email list and receive news directly, please visit http://ir.arrowheadresearch.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, actions of the U.S. Food and Drug Administration (FDA) and similar global regulatory bodies, rapid technological change in our markets, and the enforcement of our intellectual property rights. Arrowhead Research Corporation's 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 Research Corporation

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