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Arrowhead Cleared to Proceed with Multiple Dose Phase 2b Study of ARC-520

PASADENA, Calif.--(BUSINESS WIRE)-- Arrowhead Research Corporation (NASDAQ: ARWR), a biopharmaceutical company developing targeted RNAi therapeutics, today announced that the United States Food and Drug Administration (FDA) informed the Company that it can proceed with a multiple-dose Phase 2b clinical study of ARC-520, its clinical candidate for the treatment of chronic hepatitis B infection, under an investigational new drug (IND) application previously filed with the FDA.

"This notification from the FDA allows us to begin a multiple-dose Phase 2b study of ARC-520, and we are working diligently to get treatment sites up and running. Our goal is to gain site IRB approvals and perform site initiation visits to begin recruiting and enrolling patients for this study in about a month, " said Bruce Given, M.D., chief operating officer and head of R&D for Arrowhead. "We are also working with various regulatory agencies outside of the United States to initiate additional Phase 2b studies."

The clinical study, titled Heparc-2004, is a multicenter, randomized, double-blind, placebo-controlled, multi-dose study of ARC-520 administered intravenously to patients with chronic immune active HBV infection maintained on entecavir or tenofovir therapy. The study is planned to enroll up to 12 patients who will be randomized at a ratio of 2:1 with 8 patients receiving 1 mg/kg of ARC-520 and 4 patients receiving placebo. Each patient will receive 3 total doses, once every 4 weeks. Patients will be followed through Day 147.

The primary objective of Heparc-2004 is to evaluate the depth of hepatitis B surface antigen (HBsAg) decline in response to multiple doses of ARC-520 compared to placebo in patients with chronic, immune active HBV infection as a measure of drug activity. Secondary objectives are to measure safety and tolerability and pharmacokinetics of ARC-520, and to evaluate the effect of ARC-520 on entecavir or tenofovir pharmacokinetics, in addition to other exploratory safety and pharmacodynamic objectives.

In connection with an IND filed in December 2014, Arrowhead proposed a parallel design multiple-dose Phase 2b study testing doses of 2 mg/kg and 4 mg/kg simultaneously. In January 2015, the FDA notified the Company that a partial clinical hold had been placed on the program. In order to proceed with the proposed parallel design study, the FDA requested that the Company provide additional information including data from the single-dose Phase 2a study in patients who receive 1-4 mg/kg ARC-520, data from an ongoing multiple-dose non-clinical study, and data from Heparc-2004 which is now cleared to begin.

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 single dose Phase 2a studies and multiple dose Phase 2b studies 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, challenges to the validity of our intellectual property rights, 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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