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Arrowhead Begins Dosing the Phase 2b MONARCH Combination Study of ARC-520 in Patients with Chronic Hepatitis B Infection

PASADENA, Calif.--(BUSINESS WIRE)-- Arrowhead Research Corporation (NASDAQ: ARWR), a biopharmaceutical company developing targeted RNAi therapeutics, dosed the first patient in the Phase 2b MONARCH combination study of ARC-520, it's RNAi-based drug for the treatment of chronic hepatitis B virus (HBV) infection. The clinical trial is currently enrolling patients at multiple centers in Australia and New Zealand. MONARCH is intended to identify one or more combinations of ARC-520 and other therapeutic agents that can potentially lead to a functional cure of chronic HBV.

"We recently reported clinical data from our Phase 2a study at AASLD showing that a single dose of ARC-520 can achieve a reduction of up to 99% in hepatitis B surface antigen, and nonclinical data at HepDART showing that multiple doses of ARC-520 were associated with immune reactivation in 7 of 9 chimpanzees studied. These data make us very eager to see results as they emerge from MONARCH and our other multiple dose studies," said Christopher Anzalone, Ph.D., Arrowhead president and CEO. "The goal of MONARCH is to identify one or more combinations of ARC-520 and other agents that enable functional cures in patients. If we can achieve this, it would represent a big leap forward in the field and provide hope for the more than 350 million people worldwide chronically infected with the hepatitis B virus."

The MONARCH study, also called Heparc-2008, is a multicenter, open-label study to evaluate ARC-520 administered alone and in combination with other therapeutics in patients with chronic hepatitis B virus infection. The primary outcome measure is the percentage of patients achieving a 1-log reduction in hepatitis B surface antigen (HBsAg) compared to baseline. Secondary outcome measures include the percentage of patients achieving HBsAg loss and time to HBsAg loss based on qualitative assay, percentage of patients achieving anti-HBs seroconversion and time to anti-HBs seroconversion (antibody to HBsAg), percentage of patients achieving a 1-log reduction in HBsAg and achieving a HBsAg level less than 100 IU/L, as well as other outcome measures.

The initial six cohorts are each planned to consist of up to twelve treatment naïve patients. Patients in cohort 1 will receive ARC-520 as monotherapy once every 4 weeks for 48 weeks (13 doses). Patients in cohorts 2-6 will be stratified by e-antigen status and HBV genotype. They will receive the same regimen of ARC-520 for 48 weeks in combination with daily entecavir for 60 weeks and pegylated interferon alpha 2a (PEG IFN) for 48 weeks. MONARCH is designed to allow for new cohorts to be added that test additional dosing regimens and different combinations as new agents become available for study.

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 seroconversion. 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. Arrowhead is currently conducting Phase 2b multiple dose and combination studies in chronic HBV patients. In clinical studies to date, there have been no serious or severe adverse events (AEs), no dose limiting toxicities, no discontinuations due to AEs, and a modest overall occurrence rate of AEs without a clear dose-related increase in frequency or severity. The most common reported AEs in all subjects completing treatment to date were upper respiratory infection and headache.

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 and ARC-521 for chronic hepatitis B virus, ARC-AAT for liver disease associated with alpha-1 antitrypsin deficiency, ARC-F12 for hereditary angioedema and thromboembolic diseases, and ARC-HIF2 for renal cell carcinoma.

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.

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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. 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.

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