March 24, 2014

Arrowhead Begins Dosing in Phase 2a Trial of RNAi Therapeutic ARC-520 in Chronic Hepatitis B Patients

PASADENA, Calif. — March 24, 2014 — Arrowhead Research Corporation (NASDAQ: ARWR), a biopharmaceutical company developing targeted RNAi therapeutics, today announced that patient dosing has begun in a Phase 2a clinical trial of ARC-520, its RNAi therapeutic for the treatment of chronic hepatitis B virus (HBV) infection. The study is planned to enroll up to 16 chronic HBV patients in two dose cohorts with patients receiving either ARC-520 or placebo in combination with entecavir. The study is designed to evaluate the depth and duration of hepatitis B surface antigen (HBsAg) decline, among other measures, in response to a single dose of ARC-520. The Company anticipates planned enrollment to be complete in the second quarter of 2014 and expects top line results to be released in the third quarter.

“This is a significant milestone for the ARC-520 program and has broad implications for the development of additional RNAi therapeutics using the Dynamic Polyconjugate, or DPC, delivery system,” said Christopher Anzalone, Ph.D., Arrowhead’s President and CEO. “We took an important step toward clinical validation of the DPC system when the Phase 1 was completed and data indicated that ARC-520 was generally safe and well tolerated across all dose levels studied. Our next step will be confirmation that ARC-520 induces deep and durable target gene knockdown in humans. We are confident that the Phase 2a will rapidly provide us with the data necessary to make that assessment as we anticipate patient enrollment and dosing may be complete in the second quarter of this year.”

The Phase 2a study is being conducted at Queen Mary Hospital and Prince of Wales Hospital in Hong Kong. It is a multicenter, randomized, double-blind, placebo-controlled, dose-escalation study to determine the depth and duration of HBsAg reduction after a single intravenous dose of ARC-520 in combination with entecavir in patients with chronic HBV infection. Single doses of ARC-520 will be evaluated at up to two ascending doses of 1.0 mg/kg and 2.0 mg/kg. At each of the two dose levels to be evaluated, a cohort of 8 patients will be enrolled with 6 being dosed with ARC-520 and 2 being dosed with placebo. This study will be conducted in adult male and female patients aged 16 to 65 years, with immune active chronic HBV infection, HBV e antigen (HBeAg) negativity, and ongoing entecavir therapy.

The primary objective of the study is to evaluate the depth and duration of HBsAg decline in response to a single dose of ARC-520 in combination with entecavir. Secondary objectives include evaluation of safety and tolerability and pharmacokinetic (PK) measures. Additional exploratory pharmacodynamics (PD) objectives include evaluation of the effect of ARC-520 on HBV DNA serum titers and antibodies to HBsAg (anti-HBs).

Study visits will occur at screening and on days 1 (dosing), 2, 3, 8, 15, 22, 29, and 85. Patients will be monitored for HBV virology, adverse events, and exploratory PD measures for a total of 12 weeks. If HBsAg titers have not returned to within 20% of baseline by day 29 post-dose, patients will be asked to return for additional visits on days 43 and 57. The final follow up visit will occur on day 85.

About ARC-520

Arrowhead’s RNAi-based candidate ARC-520 is designed to treat chronic HBV infection by reducing the expression and release of new viral particles and key viral proteins. The goal is to achieve a functional cure, which is an immune clearant state characterized by hepatitis B s-antigen negative serum with or without sero-conversion. The siRNAs in ARC-520 intervene at the mRNA level, upstream of where nucleotide and nucleoside analogues act. In transient and transgenic mouse models of HBV infection, a single co-injection of Arrowhead’s DPC delivery vehicle with cholesterol-conjugated siRNA targeting HBV sequences resulted in multi-log knockdown of HBV RNA, proteins and viral DNA with long duration of effect. Arrowhead has completed enrollment in a Phase 1 single ascending dose study in normal volunteers. The company is conducting a single dose Phase 2a study in chronic HBV patients, which it expects to follow with a multi dose, multi national Phase 2b study. Approximately 350 million people worldwide are chronically infected with the hepatitis B virus. Chronic HBV infection 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 drug delivery technologies to develop targeted drugs based on the RNA interference mechanism that

efficiently silences disease-causing genes. Arrowhead technologies also enable partners to create peptide-drug conjugates that specifically home to cell types of interest while sparing off-target tissues. Arrowhead's pipeline includes clinical programs in chronic hepatitis B virus 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 to receive news directly, please send an email to ir@arrowres.com.

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. 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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