July 23, 2013

Arrowhead Begins Phase 1 Trial of RNAi Therapeutic ARC-520 for Treatment of Chronic Hepatitis B Infection

PASADENA, Calif. — July 23, 2013 — Arrowhead Research Corporation (NASDAQ: ARWR), a biopharmaceutical company developing targeted RNAi therapeutics, today announced that it has initiated dosing in a Phase 1 clinical trial of ARC-520, the Company's candidate for the treatment of chronic hepatitis B virus infection. Trial initiation followed successful completion of the Clinical Trial Notification (CTN) regulatory process in Australia. The objectives of the study are to characterize the safety profile of ARC-520, determine maximum tolerated dose, and evaluate pharmacokinetics in healthy volunteers. ARC-520 is the first candidate to use Arrowhead's proprietary Dynamic Polyconjugate (DPC) delivery platform and includes two distinct siRNA sequences that have pan-genotypic coverage for 99.6% HBV GenBank sequences.

The Phase 1 trial is a single-center, randomized, double-blind, placebo-controlled, single dose-escalation, first-in-human study of ARC-520 administered intravenously to healthy adult volunteers and is being conducted in Melbourne, Australia. Each dose cohort includes 6 subjects randomized at ratio of 1:2 (placebo: active) to receive a single intravenous injection of either placebo or ARC-520. Arrowhead expects to complete this Phase 1 trial in the fourth quarter of 2013 and begin a Phase 2a trial in chronic HBV patients in 2014.

"This Phase 1 study will establish a safety profile for ARC-520 as well as provide the first human data for our DPC delivery platform. This is an important step forward as we seek to advance ARC-520 into HBV patients and build additional RNAi therapeutics based on what we believe is the most potent delivery system in the industry," said Dr. Christopher Anzalone, President and Chief Executive Officer.

Hepatitis B is the world's most common serious liver infection. It is estimated that 350 million people worldwide are chronically infected with HBV, representing approximately 1 in 20 people on the planet. No currently available treatment methods can reliably achieve meaningful cure rates. ARC-520 is designed to reduce the production of new viral particles and viral proteins. Many experts believe that reducing key viral proteins can revive patients' adaptive immune response and potentially lead to a functional cure of chronic HBV infection with a finite treatment regimen. Arrowhead previously presented data generated in rodent models and in a chimpanzee chronically infected with HBV, showing that ARC-520 induces rapid, deep, and durable knockdown of both circulating HBV DNA and key viral proteins, including hepatitis B s-antigen, e-antigen, and the core protein that forms the capsid.

About ARC-520

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. 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 point of DNA transcription, 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. In a chimpanzee chronically infected with HBV and high viremia and antigenemia, ARC-520 induced rapid reductions of 90-95% in HBV DNA, e-antigen, and s-antigen. Arrowhead is conducting a phase 1 single ascending dose study in normal volunteers, which the company expects to follow with a phase 2a study in chronic HBV patients.

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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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