November 25, 2013

Arrowhead Submits Application to Begin Phase 2a Trial of ARC-520 for the Treatment of Chronic Hepatitis B Infection

PASADENA, Calif. — November 25, 2013 — Arrowhead Research Corporation (NASDAQ: ARWR), a biopharmaceutical company developing targeted RNAi therapeutics, today announced that it recently filed an application for approval to begin a phase 2a clinical trial of its RNAi-based therapeutic candidate, ARC-520, for the potential treatment of chronic hepatitis B virus infection. Pending approval, Arrowhead intends to proceed with a multicenter, randomized, double-blind, placebo-controlled, dose-escalation study. The study is being conducted to determine the depth and duration of hepatitis B surface antigen (HBsAg) reduction after a single intravenous dose of ARC-520 in combination with entecavir in patients with chronic HBV infection. Additional details on study design and anticipated timelines will be provided when patient enrollment begins.

An application for a Certificate for Clinical Trial was submitted to the Hong Kong Department of Health, and the study protocol, investigator brochure, and informed consent were submitted to the ethics committees at two sites in Hong Kong. Hong Kong was chosen as the location for the study based on the high prevalence of chronic HBV infection and for the advanced healthcare and regulatory system, which has a history of successful participation in clinical trials for antiviral agents.

Principal investigators Professor Man-Fung Yuen, Chief of Gastroenterology and Hepatology at The University of Hong Kong, Queen Mary Hospital, and Professor Henry LY Chan, Head of Gastroenterology and Hepatology at The Chinese University of Hong Kong, Prince of Wales Hospital, will conduct the study. These investigators and sites were selected based on their large pool of patients currently under care, their international standing as HBV researchers, and track record of high accrual rates for clinical trials involving viral hepatitis.

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 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. 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, which did not return to baseline until study day 43, 43, and 71 respectively. Data also suggested that a therapeutic immunological flare occurred, which is thought to be part of a cascade that under chronic therapy may lead to HBsAg seroconversion and functional cure. Arrowhead has completed enrollment in 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.

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 silence 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 obesity and partner-based programs in 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

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