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Arrowhead to Present ARC-520 Phase 2a Data in Late-Breaking Session at AASLD Liver Meeting® 2014

PASADENA, Calif.--(BUSINESS WIRE)-- Arrowhead Research Corporation (NASDAQ: ARWR), a biopharmaceutical company developing targeted RNAi therapeutics, today announced that data from the ongoing Phase 2a study of ARC-520, its RNAi therapeutic candidate for the treatment of chronic hepatitis B (HBV) infection, will be presented in the late-breaking poster session at the 2014 American Association for the Study of Liver Diseases (AASLD) Liver Meeting being held on November 7-11, 2014, in Boston. Arrowhead was also selected to deliver a plenary presentation with new preclinical efficacy data on ARC-AAT, its RNAi therapeutic candidate for the treatment of liver disease associated with Alpha-1 antitrypsin deficiency. Additional details including abstracts for both presentations can be found in The Liver Meeting section of the AASLD website at http://www.aasld.org/livermeeting/Pages/default.aspx.

"ARC-520 represents a novel approach for the treatment of HBV with the potential to achieve functional cures," said Christopher Anzalone, Ph.D., Arrowhead's President and Chief Executive Officer. "Our ongoing Phase 2a dose finding study is an important step, and in cohort 1 at a dose of 1 mg/mg and cohort 2 at 2 mg/kg we saw a clear reduction in HBsAg, the surface antigen of HBV. Data collection for HBsAg reduction in cohort 3 at 3 mg/kg is still ongoing, however we are pleased to report that all three dose levels have been well tolerated in patients. These results give us great confidence as we move forward with designing and initiating several upcoming Phase 2b studies of ARC-520 and the ARC-AAT Phase 1 study."

Arrowhead presentations can be attended during the following times:

9:15 a.m. EST, Nov. 10, Plenary Session, John B. Hynes Convention Center, Auditorium - An oral presentation titled, "**A hepatocyte-targeted RNAi-based treatment for liver disease associated with alpha-1 antitrypsin deficiency**," will be presented by Christine Wooddell, Ph.D., Group Leader, Arrowhead Research, Madison, Wis.

8 a.m. to 5:30 p.m. EST, Nov. 10, Late-Breaking Poster Session, John B. Hynes Convention Center, Hall C - A poster presentation titled, "**Phase II, dose ranging study of ARC-520, a siRNA-based therapeutic, in patients with chronic hepatitis B virus infection**," will be presented by Man-Fung Yuen, M.D., Ph.D., Chair of Gastroenterology and Hepatology, and Li Shu Fan Medical Foundation Professor in Medicine, The University of Hong Kong.

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 Dynamic Polyconjugate (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, and expects to follow with multi-dose, multi-national Phase 2b studies. 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 ARC-AAT

Arrowhead has developed ARC-AAT for the treatment of liver disease associated with Alpha-1 Antitrypsin Deficiency (AATD), a rare genetic disease that severely damages the liver and lungs of affected individuals. ARC-AAT employs a novel unlocked nucleobase analog (UNA) containing RNAi molecule designed for systemic delivery using the Dynamic Polyconjugate delivery system. ARC-AAT is highly effective at knocking down the Alpha-1 antitrypsin (AAT) gene transcript and reducing the hepatic production of mutant AAT (Z-AAT) protein. Reduction of inflammatory Z-AAT protein, which has been clearly defined as the cause of progressive liver disease in AATD patients, is important as it is expected to halt the progression of liver disease and allow fibrotic tissue repair. The Company plans to file an Investigational New Drug (IND) or equivalent application for ARC-AAT in the fourth quarter of 2014 and commence clinical studies shortly thereafter.

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.

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