November 16, 2015

Arrowhead Late-Breaking Clinical Data Shows that ARC-520 Can Produce Deep and Durable Reductions of Hepatitis B Viral Antigens and DNA

PASADENA, Calif.--(BUSINESS WIRE)-- Arrowhead Research Corporation (NASDAQ: ARWR), a biopharmaceutical company developing targeted RNAi therapeutics, presented data from a Phase 2a clinical study at The AASLD Liver Meeting 2015® demonstrating that ARC-520, its lead drug candidate against chronic hepatitis B infection (HBV), effectively reduced HBV viral antigens derived from cccDNA. HBV surface antigen (HBsAg) was reduced substantially with a maximum reduction of 1.9 logs (99%) and a mean maximum reduction of 1.5 logs (96.8%) in treatment naïve e-antigen (HBeAg)-positive patients. This direct antiviral effect was still evident 57 days after a single dose. These data strongly support advancement of ARC-520, and Arrowhead has initiated multiple studies aimed at producing a functional cure of HBV.

Christopher Anzalone, Ph.D., Arrowhead's president and chief executive officer said, "At AASLD we presented data from our clinical program and from a nonclinical study in chimpanzees. Both of these studies show that ARC-520 can produce deep and durable knockdown of HBV viral antigens. These data give us additional confidence in the program as we move forward with multiple-dose and combination studies of ARC-520, that we hope will lead to host immune reconstitution, HBsAg seroclearance, and functional cure."

Man-Fung Yuen, M.D., Ph.D., chair of gastroenterology and hepatology, The University of Hong Kong, and deputy chief of service, Queen Mary Hospital department of medicine, Hong Kong, and a principal investigator for Arrowhead's Phase 2a clinical study, delivered a late-breaking poster presentation titled, "**ARC-520 produces deep and durable knockdown of viral antigens and DNA in a phase II study in patients with chronic hepatitis B**".

In this presentation, Dr. Yuen and co-authors show that in the Heparc-2001 clinical study, ARC-520 in combination with entecavir achieved maximum reductions of HBsAg, HBV DNA, HBeAg, and core-related antigen (HBcrAg) of 1.9 logs (99%), 4.3 logs (99.995%), 1.7 logs (98%), and 1.2 logs (93.7%), respectively.

HBeAg-positive, treatment naïve patients achieved consistent reductions in HBsAg with a mean maximal reduction of 1.5 logs (96.8%). ARC-520 caused a direct antiviral effect after a single dose that was still evident after 57 days, which was the last time-point available.

Consistent with findings from Arrowhead's chimpanzee study, also presented at AASLD, variations in viral antigen reduction indicated that patients previously treated with chronic entecavir and patients that were treatment-naive and negative for HBeAg likely had lower levels of cccDNA derived mRNA transcripts. As such, HBeAg-positive treatment naïve patients experienced a greater relative reduction in HBsAg than patients that were HBeAg-negative or treatment experienced. One transitional patient in cohort 7 was HBeAg-positive at baseline and became HBeAg-negative at days 3 to 43. This patient experienced an intermediate response initially, however HBsAg continued to trend downward through day 57, the last time-point available.

In the clinical study, 58 patients with chronic HBV received doses of 1mg/kg - 4 mg/kg of ARC-520 in 7 cohorts. The cohorts varied by ARC-520 dose, HBeAg status, and prior NUC treatment status. The primary objective of the study was to measure the depth and duration of HBsAg reduction in response to a single dose or two doses (cohort 6) of ARC-520 in combination with entecavir. Arrowhead also assessed safety and tolerability and additional secondary and exploratory endpoints.

ARC-520 was well tolerated with no serious adverse events (AE), no dose limiting toxicities, no discontinuations due to medication AEs, and a modest occurrence rate (23%) of AEs that were all deemed unrelated to study drug by the principal investigator. No AE occurred more than once. There were no AEs amongst 10 patients receiving placebo. There was a low occurrence rate of abnormal laboratory tests, with no observed relationship to timing or dose.

Copies of presentation materials can be accessed by visiting the Events section of the company's website at http://ir.arrowheadresearch.com/events.cfm.

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. Arrowhead is conducting Phase 2b multiple dose and combination studies in chronic HBV patients. 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.

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.

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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