January 12, 2015

Arrowhead Provides Update on IND for ARC-520 Phase 2b Study

PASADENA, Calif.--(BUSINESS WIRE)-- Arrowhead Research Corporation (NASDAQ: ARWR), a biopharmaceutical company developing targeted RNAi therapeutics, today announced that the U.S. Food and Drug Administration (FDA) verbally informed the Company in a preliminary call of a partial clinical hold, under which the Company is cleared to begin a modified multiple-dose study of ARC-520 in patients with chronic hepatitis B infection. The FDA requested that the Company start the multiple-dose study at 1 mg/kg of ARC-520 rather than the proposed parallel study design of 2 and 4 mg/kg, and requested additional information be provided to the agency. The additional information includes a final study report from the single-dose Phase 2a study in patients who received 1-4 mg/kg ARC-520, which is ongoing and has not reported any serious adverse events or evidence of end organ toxicity to date. The FDA also requested a final study report from an ongoing multiple-dose non-clinical study, which has shown ARC-520 to be well tolerated with no evidence of end organ toxicity to date. The FDA committed to provide the Company with a letter detailing its thoughts and requests within 30 days. The ongoing Phase 2a study continues as planned, and the Company expects to file with Asian and European agencies to begin additional Phase 2b studies in coming weeks.

"Over the next 30 days, Arrowhead will begin preparations for the multiple-dose Phase 2b study," said Arrowhead President and CEO, Dr. Christopher Anzalone. "We will work closely with the FDA throughout this process while we continue to seek approval to proceed with other planned studies in Asia and Europe."

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 sero-conversion. Arrowhead has completed a Phase 1 single ascending dose study in normal volunteers and the company is conducting a single dose Phase 2a study 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 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.

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, actions of the U.S. Food and Drug Administration (FDA) and similar global regulatory bodies, 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.

Source: Arrowhead Research Corporation

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