December 9, 2013

Arrowhead Presents Phase 1 Data on ARC-520 at HepDART 2013

PASADENA, Calif. — December 9, 2013 — Arrowhead Research Corporation (NASDAQ: ARWR), a biopharmaceutical company developing targeted RNAi therapeutics, today announced that COO and Head of R&D, Bruce Given, M.D., presented data on the Phase 1 clinical study of ARC-520, the company's clinical candidate for the treatment of chronic hepatitis B infection, at the HepDART 2013 conference being held on The Big Island, Hawaii. New data including pharmacokinetics (PK) and adverse event (AE) attribution presented today in a poster and in an oral presentation tomorrow, support the previous findings that ARC-520 appears to be generally safe and well-tolerated at all six dose levels studied.

The Phase 1 study was designed to characterize the safety profile of ARC-520 across a range of doses and evaluate pharmacokinetics. It 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. 36 subjects have been enrolled in 6 groups randomized at a ratio of 2:1 to receive ARC-520 or placebo: Placebo (n=12), ARC-520 0.01 mg/kg (n=4), 0.1 mg/kg (n=4), 0.3 mg/kg (n=4), 0.6 mg/kg (n=4), 1.2 mg/kg (n=4), and 2.0 mg/kg (n=4). The placebo group included 7 male and 5 female subjects with average of 28.1 +/- 9.6 years. The treatment group included 12 male and 12 female subjects with average age of 26.9 +/- 6.7 years. Subjects were admitted to the unit overnight pre-dose and vital signs, telemetry, ECGs, safety labs, PK, and adverse events were monitored for 24 hours post-dose. Return visits occurred for repeat safety evaluations and recording of adverse events at 48 hrs, 72 hours, day 7, day 14 and day 28 post dosing.

Preliminary results from the phase 1 clinical study of ARC-520 indicate that to date there have been no serious AEs, no dose limiting toxicities, no discontinuations, and a modest occurrence rate of AEs with no dose related increase in frequency or severity, with the possible exception of mild lightheadedness which occurred in two subjects in the 2 mg/kg dose group. There were no general differences observed or findings rated clinically significant on vital signs, ECGs, physical examinations, or clinical laboratories in the ARC-520 groups relative to placebo. Adverse event frequency and severity did not differ between placebo and ARC-520, with 75% of both treated and placebo subjects reporting mild or moderate AEs. There was a low occurrence rate of abnormal laboratory tests, with no observed relationship to timing or dose. PK results appear to indicate that C_0 (equivalent to C_{Max}) and $AUC_{0-\infty}$ increase linearly with dose (r^2=0.984).

The Phase 1 study has thus demonstrated that a single intravenous administration of ARC-520 appears to be safe and well tolerated up to and including a dose of 2 mg/kg, the highest dose tested. A copy of the poster presentation is available on the Presentations and Events page of Arrowhead website at http://www.arrowheadresearch.com/presentations.

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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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