

December 18, 2013

# Arrowhead Reports Fiscal 2013 Fourth Quarter and Year-End Financial Results

### Conference Call Today at 4:30 p.m. Eastern Time

PASADENA, Calif. — December 18, 2013 — Arrowhead Research Corporation (NASDAQ: ARWR), a biopharmaceutical company developing targeted RNAi therapeutics, today announced financial results for its fiscal 2013 fourth quarter and year ended September 30, 2013. The company is hosting a conference call at 4:30 p.m. Eastern time to discuss results. To participate, please dial 877-300-8521 (toll free from the US), 855-669-9657 (toll free from Canada), or 412-317-6026 (for international callers). Investors may also access a live audio webcast of this conference call on the Company's website at http://www.arrowheadresearch.com/presentations.

A replay of the webcast will be available approximately one hour after the conclusion of the call and will remain available for 90 days. An audio replay will also be available approximately two hours after the conclusion of the call and will be available for 30 days. The audio replay can be accessed by dialing 877-870-5176 (toll free from the US and Canada), or 858-384-5517 (for international callers), and entering Event ID 10038065.

## Fiscal 2013 Fourth Quarter and Recent Company Highlights

- Closed a \$60 million financing from a syndicate of high-quality biotech investors. This provided sufficient capital to fund development into 2016;
- Presented additional data at the AASLD Liver Meeting on the ARC-520 chimpanzee study indicating possible immune derepression as hepatitis B s-antigen levels were reduced;
- Completed planned enrollment of a Phase 1 clinical trial of ARC-520 in healthy volunteers;
- Submitted an application for a Certificate of Clinical Trial to the Hong Kong Department of Health to begin a Phase 2a clinical trial of ARC-520 in chronic HBV patients; and
- Presented Phase 1 data at HepDART 2013 showing that ARC-520 was generally safe and well-tolerated at all six dose levels studied.

#### Selected Fiscal 2013 Year End Financial Results

Total operating expenses for the year ended September 30, 2013 were \$24.9 million, compared to 21.2 million for the year ended September 30, 2012.

Net loss attributable to Arrowhead for the year ended September 30, 2013 was \$31.1 million, or \$1.30 per share based on 24 million weighted average shares outstanding. This compares with a net loss attributable to Arrowhead of \$21.1 million, or \$1.90 per share based on 11.1 million weighted average shares outstanding, for the year ended September 30, 2012.

Net cash used in operating activities in fiscal 2013 was \$19 million, compared with \$15.3 million in the prior year period. The increase in operating expenses and cash used in operating activities of approximately \$3.5 million, as compared to the prior fiscal year, reflects final pre-clinical requirements, including GMP manufacturing and GLP toxicology, to enable our HBV candidate, ARC-520, to enter clinical trials, as well as expenditures related to the phase 1 trial of ARC-520 for which we completed planned enrollment in October 2013.

The company's cash and investments of cash were \$29.8 million at September 30, 2013, compared to \$3.4 million at September 30, 2012. The increase in cash balance reflects \$42.5 million in cash from financings during fiscal 2013, plus cash inflow from warrant exercises of \$2 million. Subsequent to September 30, 2013, an additional financing that closed in October 2013 yielded \$60 million in net proceeds.

During the fiscal year, cash outlays for research and development were \$13 million, and cash used for general and administrative purposes was \$6.6 million. Cash inflows during the fiscal year included \$42.5 million from the sale of equity securities, \$2 million from warrant exercises, \$300,000 in revenue, and \$1.4 million in proceeds related to the sale of our former subsidiary, Unidym.

Common shares outstanding at September 30, 2013, were 32.5 million, and 37.9 million assuming conversion of preferred shares outstanding at September 30, 2013. Taking into account the October financing and shares issued through the exercise of warrants since the end of the fiscal year, current common shares outstanding are 38.7 million, and 49.4 million assuming conversion of preferred shares outstanding.

#### 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 partner-based programs in obesity and oncology.

For more information please visit <a href="http://www.arrowheadresearch.com">http://www.arrowheadresearch.com</a>, or follow us on Twitter <a href="mailto:@ArrowRes">@ArrowRes</a>. To be added to the Company's email list to receive news directly, please send an email to <a href="mailto:ir@arrowres.com">ir@arrowres.com</a>.

### 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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