August 12, 2014

Arrowhead Reports Fiscal 2014 Third Quarter Financial Results and Provides Update on ARC-520

- Conference Call Today at 4:30 p.m. EDT

PASADENA, Calif.--(BUSINESS WIRE)-- Arrowhead Research Corporation (NASDAQ: ARWR), a biopharmaceutical company developing targeted RNAi therapeutics, today announced financial results for its fiscal 2014 third quarter ended June 30, 2014 and provided an update on the Phase 2a study of ARC-520, its RNAi-based candidate for the treatment of chronic hepatitis B infection. The company is hosting a conference call at 4:30 p.m. EDT to discuss results. Conference call and webcast details can be found below.

ARC-520 Phase 2a Study Update

- Completed dosing of 1 mg/kg and 2 mg/kg dose cohorts
- Initial blinded data suggest that the magnitude of HBsAg knockdown is similar to non-human primate studies, including the chronically infected chimpanzee reported on previously
- Duration of knockdown appears to be substantially more sustained than in non-human primates, with patients in the 2 mg/kg group still demonstrating substantial knockdown after 8 weeks, which is the most recent time point available
- HBsAg levels appear to continue to decline in a number of patients at the 8 week time point in the 2 mg/kg group
- Based on initial review, dosing less frequent than once monthly will be explored in Phase 2b
- ARC-520 continues to be well tolerated, with no dropouts or serious adverse events reported
- The overall rate of AEs has been lower in the Phase 2a than in the Phase 1 normal volunteer study and safety labs continue to show no indication of end organ toxicity
- Enrolled and dosed additional subjects at 3 mg/kg in the still open normal volunteer study and the dose performed well, without detected differences from safety and tolerability results at the other doses. Overall AEs do not appear to be increasing in frequency or severity with dose
- Received IRB and DSMB approvals to proceed and began enrolling an additional dose cohort at 3 mg/kg in the Phase 2a patient study

Fiscal 2014 Third Quarter and Recent Company Highlights

- Nominated a second clinical candidate using our DPC delivery system, ARC-AAT, for the treatment of a rare liver disease associated with alpha-1 antitrypsin deficiency and hosted an analyst day to present preclinical data
- Signed an agreement with The Alpha-1 Project (TAP), the venture philanthropy subsidiary of the Alpha-1 Foundation. Under the terms of the agreement, TAP will partially fund the development of ARC-AAT. In addition, TAP will make its scientific advisors available to Arrowhead, assist with patient recruitment for clinical trials through the Alpha-1 Foundation Patient Research Registry, and engage in other collaborative efforts that support the development of ARC-AAT
- Initiated the final steps required to file an IND or equivalent application for ARC-AAT, including necessary toxicology studies
- Expanded intellectual property protection with U.S. Patent Application number 13/535,454 covering ARC-520's siRNA component, being recently allowed by the U.S. Patent and Trademark Office
- Presented data on advancements made to the DPC delivery system at multiple scientific meetings
- Arrowhead added to the broad-market Russell 3000 index and small-cap Russell 2000 index

Selected Fiscal 2014 Third Quarter Financial Results

Net loss attributable to Arrowhead for the quarter was $11.6 million, or $0.22 per share based on 51.9 million weighted average shares outstanding. This compares with a net loss attributable to Arrowhead of $6.1 million, or $0.23 per share based on 26.1 million weighted average shares outstanding, for the quarter ended June 30, 2013.

Total operating expenses for the quarter were $12.7 million, compared to $6.4 million for the quarter ended June 30, 2013. Research and development related expenses were $6.4 million and general and administrative expenses were $1.6 during the quarter.

Net cash used in operating activities for the first nine months of fiscal 2014 was $24.5 million, compared with $13.6 million in the prior year period.

The company's cash and investments of cash were $188.5 million at June 30, 2014, compared to $29.8 million at September 30, 2013. The increase in the cash balance reflects financings completed in October 2013 and February 2014, plus cash inflow from exercise of warrants and stock options of $12.4 million.

Common shares outstanding at June 30, 2014, were 52.9 million, and 58.5 million assuming conversion of preferred stock outstanding.

Conference Call and Webcast Details

To participate in the conference call, please dial 855-215-6159 (toll free from the US) or 315-625-6887 (for international callers) and enter Conference ID 82825377. Investors may also access a live audio webcast of this conference call on the Company's website at http://ir.arrowheadresearch.com/events.cfm.

A replay of the webcast will be available approximately two hours 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 7 days. The audio replay can be accessed by dialing 855-859-2056 (toll free from the US), or 404-537-3406 (for international callers) and entering Conference ID 82825377.

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. The company is conducting a single dose Phase 2a study in chronic HBV patients, and expects to follow with a multi-dose, multi-national Phase 2b program. 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 for regulatory permission in the fourth quarter of 2014 and to commence clinical studies.

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 to receive news directly, please send an email to ir@arrowres.com

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.

Arrowhead Research Corporation
Vince Anzalone, CFA
626-304-3400
ir@arrowres.com
or
Investor Relations:
The Trout Group
Lauren Glaser
646-378-2972
ir@arrowres.com
or
Media:
Russo Partners
Martina Schwarzkopf, Ph.D.
212-845-4292
martina.schwarzkopf@russopartnersllc.com

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