

November 25, 2014

# **Arrowhead Reports Fiscal 2014 Year End Financial Results**

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

PASADENA, Calif.--(BUSINESS WIRE)-- Arrowhead Research Corporation (NASDAQ: ARWR), a biopharmaceutical company developing targeted RNAi therapeutics, today announced financial results for its fiscal 2014 fourth quarter and year ended September 30, 2014. The company is hosting a conference call at 4:30 p.m. EST to discuss results.

#### **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 38229976. Investors may also access a live audio webcast of this conference call on the Company's website at <a href="http://ir.arrowheadresearch.com/events.cfm">http://ir.arrowheadresearch.com/events.cfm</a>.

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 38229976.

# Fiscal 2014 Year End Company Highlights

#### Corporate Highlights

- Strengthened our balance sheet with equity financings totaling approximately \$172.6 million in net proceeds during fiscal 2014
- Hired additional R&D staff in key areas, including manufacturing, toxicology, chemistry, biology, quality assurance, regulatory and clinical operations to support rapid development of ARC-520 and additional clinical candidates
- Upgraded NASDAQ listing to the Global Select Market
- Joined the broad-market Russell 3000 Index and the small-cap Russell 2000 Index

## Pipeline Highlights

- Nominated ARC-AAT as the Company's next clinical candidate and held an analyst day in June 2014 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
- Completed planned enrollment of six initial dose cohorts (n=36) and three additional cohorts (n=18) in a Phase 1 clinical trial indicating ARC-520 was generally well-tolerated in all nine cohorts at ascending dose levels of 0.01 4.0 mg/kg
- Initiated a Phase 2a dose finding study of ARC-520 and completed dosing of three initial dose cohorts (n=24) at 1.0, 2.0, and 3.0 mg/kg, with a 4.0 mg/kg cohort now underway
- Presented preclinical data on ARC-520 at the 2013 AASLD Liver Meeting demonstrating reduction of key HBV antigens and DNA, and evidence of immune reactivation in a chimpanzee with chronic HBV infection
- Presented interim data on the ongoing Phase 2a study of ARC-520 and additional preclinical data on ARC-AAT at the 2014 AASLD Liver Meeting

#### Selected Fiscal 2014 Year End Financial Results

# ARROWHEAD RESEARCH CORPORATION CONSOLIDATED CONDENSED FINANCIAL INFORMATION

OPERATING SUMMARY	Year Ended S	September 30, 2013
REVENUE	\$ 175,000	\$ 290,266
OPERATING EXPENSES		
Research and development	23,138,050	8,705,627
Salaries and payroll-related costs	12,829,355	6,667,669
General and administrative expenses	5,894,008	3,488,864
Stock-based compensation	5,696,173	1,536,271
Depreciation and amortization	1,345,655	1,751,412
Impairment expense	2,172,387	1,308,047
Contingent consideration - fair value adjustments	2,375,658	1,421,652
TOTAL OPERATING EXPENSES	53,451,286	24,879,542
OPERATING LOSS	(53,276,286)	(24,589,276)
OTHER INCOME/(EXPENSE), LOSS FROM DISCONTINUED OPERATIONS, PROVISION FOR		
INCOME TAXES	(5,449,126)	· <del></del>
NET LOSS	\$ (58,725,412)	\$(31,703,433)
EARNINGS PER SHARE (BASIC AND DILUTED):	\$ (1.25)	\$ (1.30)
WEIGHTED AVERAGE SHARES OUTSTANDING	46,933,030	24,002,224
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FINANCIAL POSITION SUMMARY	September 30,	
	2014	2013
CASH AND CASH EQUIVALENTS	132,510,610	19,114,444
SHORT AND LONG-TERM INVESTMENTS	44,741,378	
TOTAL CASH RESOURCES (CASH, CASH EQUIVALENTS AND INVESTMENTS)	177,251,988	29,846,858
OTHER ASSETS	5,564,768	7,482,773
TOTAL ASSETS	182,816,756	37,329,631
TOTAL LIABILITIES	16,831,501	11,594,842
TOTAL STOCKHOLDERS' EQUITY	165,985,255	25,734,789
TOTAL LIABILITIES AND STOCKHOLDERS' EQUITY	182,816,756	37,329,631
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SHARES OUTSTANDING	54,656,936	32,489,444
PROFORMA SHARES OUTSTANDING (INCLUDING CONVERSION OF PREFERRED	34,030,330	32,703,777
SHARES)	58,644,142	37,899,280

#### 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. Arrowhead has completed enrollment in a Phase 1 single ascending dose study in normal volunteers. The company is conducting a single dose Phase 2a study in chronic HBV patients, and expects to follow with multi-dose, multi-national Phase 2b studies. Approximately 350-400 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 trigger 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 the mutant AAT (Z-AAT) protein in animal models. Reduction of liver production of the 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 potentially allow fibrotic tissue repair. The Company filed to initiate testing in humans in the fourth quarter of 2014 and intends to begin the study shortly after receiving approval.

#### **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, 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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