

February 9, 2015

# Arrowhead Reports Fiscal 2015 First Quarter 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 2015 first quarter ended December 31, 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 76024600. 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 76024600.

## Fiscal 2015 First Quarter and Recent Company Highlights

- Expanded the management team with the appointment of Susan Boynton as Vice President, Regulatory Affairs and Patrick O'Brien as General Counsel
- Presented initial data from the ongoing Phase 2a study of ARC-520, our RNAi therapeutic candidate for the treatment of chronic hepatitis B infection, in the late-breaking poster session at the 2014 American Association for the Study of Liver Diseases Meeting (AASLD)
- Delivered a plenary presentation at AASLD with new preclinical efficacy data on ARC-AAT, our RNAi therapeutic candidate for the treatment of liver disease associated with Alpha-1 antitrypsin deficiency
- Filed an application for approval to begin a Phase 1 clinical trial of ARC-AAT
- Submitted an IND application to the U.S. Food and Drug Administration to begin Phase 2b multiple dose studies
- Completed dosing of 3 & 4 mg/kg cohorts in Phase 2a study of ARC-520

## Selected Fiscal 2015 First Quarter Financial Results

# ARROWHEAD RESEARCH CORPORATION CONSOLIDATED CONDENSED FINANCIAL INFORMATION

OPERATING SUMMARY	Three Months Ended December 31,	
	2014	2013
REVENUE	\$170,750	\$43,750
OPERATING EXPENSES		
Research and development	17,744,312	3,133,014
Salaries and payroll-related costs	3,150,617	2,081,791
General and administrative expenses	2,086,202	913,784
Stock-based compensation	2,014,856	521,138
Depreciation and amortization	290,039	403,405
TOTAL OPERATING EXPENSES	25,286,026	7,053,132

OPERATING LOSS	(25,115,276)	(7,009,382)
OTHER INCOME/(EXPENSE)	2,539,994	(3,676,350)
NET LOSS	\$(22,575,282)	\$(10,685,732)
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EARNINGS PER SHARE (BASIC AND DILUTED):	\$(0.41)	\$(0.28)
WEIGHTED AVERAGE SHARES OUTSTANDING	54,692,392	37,741,743
	December 31,	September 30,
FINANCIAL POSITION SUMMARY	2014	2014
CASH AND CASH EQUIVALENTS	103,991,231	132,510,610
SHORT AND LONG-TERM INVESTMENTS	41,338,901	44,741,378
TOTAL CASH RESOURCES (CASH, CASH EQUIVALENTS AND INVESTMENTS)	145,330,132	177,251,988
OTHER ASSETS	16,806,629	5,564,768
TOTAL ASSETS	162,136,761	182,816,756
TOTAL LIABILITIES	16,427,090	16,831,501
TOTAL STOCKHOLDERS' EQUITY	145,709,671	165,985,255
TOTAL LIABILITIES AND STOCKHOLDERS' EQUITY	162,136,761	182,816,756
SHARES OUTSTANDING	54,715,714	54,656,936
PROFORMA SHARES OUTSTANDING (INCLUDING CONVERSION OF PREFERRED		
SHARES)	58,702,920	58,644,142

## 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 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. 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 with regulatory authorities in the fourth quarter of 2014 and intends to initiate clinical studies shortly after receiving permission to begin.

## 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 <u>http://www.arrowheadresearch.com</u>, or follow us on Twitter <u>@ArrowRes</u>. To be added to the Company's email list and receive news directly, please visit <u>http://ir.arrowheadresearch.com/alerts.cfm</u>.

## 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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Source: Arrowhead Research Corporation

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