

May 11, 2015

Arrowhead Reports Fiscal 2015 Second Quarter Financial Results

- Conference Call and Webcast 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 2015 second quarter ended March 31, 2015. The company is hosting a conference call at 4:30 p.m. EDT 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 40592020. 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 40592020.

Fiscal 2015 Second Quarter and Recent Company Highlights

- Started phase 1 study of ARC-AAT, the company's clinical candidate against liver disease associated with alpha-1 antitrypsin deficiency
- Completed dosing of Part A of the ARC-AAT phase 1 study in healthy volunteers, and transitioned the study into Part B
 which will enroll patients with PiZZ genotype alpha-1 antitrypsin deficiency
- Completed the acquisition of Novartis' RNAi research and development portfolio, related licenses, and assets
- Gained clearance from the U.S. Food and Drug Administration to begin the Heparc-2004 multi-dose Phase 2b study of ARC-520
- Filed with various regulatory authorities in Europe and Asia to explore additional multi-dose studies of ARC-520 outside of the U.S.
- Completed dosing in two additional dose cohorts in the Heparc-2001, a single-dose phase 2a study of ARC-520
- Expanded Heparc-2001 to include three additional cohorts, which will be discussed on the call at 4:30 p.m. EDT
- Published data on a new construct of the Dynamic Polyconjugate delivery system that appears to have better stability and longer circulation times, which may enable subcutaneous administration and potentially extra-hepatic targeting
- Presented preclinical data on ARC-F12, a potential new candidate targeting coagulation factor 12 for the potential treatment of hereditary angioedema and thromboembolic diseases

Selected Fiscal 2015 Second Quarter Financial Results

ARROWHEAD RESEARCH CORPORATION CONSOLIDATED CONDENSED FINANCIAL INFORMATION (unaudited)

	Three Months Ended March 31,		Six Months Ended March 31,		
OPERATING SUMMARY	 2015	2014	 2015		2014
REVENUE	\$ 43,750 \$	43,750	\$ 214,500	\$	87,500

OPERATING EXPENSES				
Research and development	11,640,794	5,216,446	29,387,524	8,349,460
Acquired in-process research and development	10,142,786	-	10,142,786	-
Salaries and payroll-related costs	3,541,652	3,097,902	6,692,268	5,179,693
General and administrative expenses	1,696,623	1,347,677	3,782,826	2,261,461
Stock-based compensation	2,205,079	1,198,444	4,219,935	1,719,582
Depreciation and amortization	449,559	395,779	739,598	799,184
TOTAL OPERATING EXPENSES	29,676,493	11,256,248	54,964,937	18,309,380
OPERATING LOSS	(29,632,743)	(11,212,498)	(54,750,437)	(18,221,880)
OTHER INCOME/(EXPENSE)	948,750	(2,770,202)	3,488,743	(6,446,551)
NET LOSS	\$ (28,683,993)	\$ (13,982,700)	\$(51,261,694)	\$(24,668,431)
EARNINGS PER SHARE (BASIC AND DILUTED):	<u>\$ (0.51</u>)	<u>\$ (0.31)</u>	\$ (0.93)	<u>\$ (0.60</u>)
WEIGHTED AVERAGE SHARES OUTSTANDING	55,719,923	44,321,847	55,200,512	40,941,903
FINANCIAL POSITION SUMMARY	March 31,	December 31,		
FINANCIAL POSITION SUMMARY	2015	2014		
FINANCIAL POSITION SUMMARY CASH AND CASH EQUIVALENTS				
	2015	2014		
CASH AND CASH EQUIVALENTS SHORT AND LONG-TERM INVESTMENTS TOTAL CASH RESOURCES (CASH, CASH EQUIVALENTS AND	2015 96,447,301 31,922,260	2014 103,991,231 41,338,901		
CASH AND CASH EQUIVALENTS SHORT AND LONG-TERM INVESTMENTS TOTAL CASH RESOURCES (CASH, CASH EQUIVALENTS AND INVESTMENTS)	2015 96,447,301 31,922,260 128,369,561	2014 103,991,231 41,338,901 145,330,132		
CASH AND CASH EQUIVALENTS SHORT AND LONG-TERM INVESTMENTS TOTAL CASH RESOURCES (CASH, CASH EQUIVALENTS AND	2015 96,447,301 31,922,260 128,369,561 34,008,897	2014 103,991,231 41,338,901 145,330,132 16,806,629		
CASH AND CASH EQUIVALENTS SHORT AND LONG-TERM INVESTMENTS TOTAL CASH RESOURCES (CASH, CASH EQUIVALENTS AND INVESTMENTS)	2015 96,447,301 31,922,260 128,369,561	2014 103,991,231 41,338,901 145,330,132		
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CASH AND CASH EQUIVALENTS SHORT AND LONG-TERM INVESTMENTS TOTAL CASH RESOURCES (CASH, CASH EQUIVALENTS AND INVESTMENTS) OTHER ASSETS TOTAL ASSETS	2015 96,447,301 31,922,260 128,369,561 34,008,897 162,378,458	2014 103,991,231 41,338,901 145,330,132 16,806,629 162,136,761		
CASH AND CASH EQUIVALENTS SHORT AND LONG-TERM INVESTMENTS TOTAL CASH RESOURCES (CASH, CASH EQUIVALENTS AND INVESTMENTS) OTHER ASSETS TOTAL ASSETS TOTAL LIABILITIES	2015 96,447,301 31,922,260 128,369,561 34,008,897 162,378,458 18,182,104	2014 103,991,231 41,338,901 145,330,132 16,806,629 162,136,761 16,427,090		
CASH AND CASH EQUIVALENTS SHORT AND LONG-TERM INVESTMENTS TOTAL CASH RESOURCES (CASH, CASH EQUIVALENTS AND INVESTMENTS) OTHER ASSETS TOTAL ASSETS TOTAL LIABILITIES TOTAL STOCKHOLDERS' EQUITY	2015 96,447,301 31,922,260 128,369,561 34,008,897 162,378,458 18,182,104 144,196,354	2014 103,991,231 41,338,901 145,330,132 16,806,629 162,136,761 16,427,090 145,709,671		
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CASH AND CASH EQUIVALENTS SHORT AND LONG-TERM INVESTMENTS TOTAL CASH RESOURCES (CASH, CASH EQUIVALENTS AND INVESTMENTS) OTHER ASSETS TOTAL ASSETS TOTAL LIABILITIES TOTAL STOCKHOLDERS' EQUITY TOTAL LIABILITIES AND STOCKHOLDERS' EQUITY SHARES OUTSTANDING PROFORMA SHARES OUTSTANDING (INCLUDING	2015 96,447,301 31,922,260 128,369,561 34,008,897 162,378,458 18,182,104 144,196,354 162,378,458	2014 103,991,231 41,338,901 145,330,132 16,806,629 162,136,761 16,427,090 145,709,671 162,136,761 54,715,714		
CASH AND CASH EQUIVALENTS SHORT AND LONG-TERM INVESTMENTS TOTAL CASH RESOURCES (CASH, CASH EQUIVALENTS AND INVESTMENTS) OTHER ASSETS TOTAL ASSETS TOTAL LIABILITIES TOTAL STOCKHOLDERS' EQUITY TOTAL LIABILITIES AND STOCKHOLDERS' EQUITY SHARES OUTSTANDING	2015 96,447,301 31,922,260 128,369,561 34,008,897 162,378,458 18,182,104 144,196,354 162,378,458	2014 103,991,231 41,338,901 145,330,132 16,806,629 162,136,761 16,427,090 145,709,671 162,136,761		

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 single dose Phase 2a studies and multiple dose Phase 2b studies 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's ARC-AAT is being investigated 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 is likely a 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 is conducting a single dose Phase 1 clinical study, with part A in healthy volunteers and part B in AATD patients.

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 and ARC-AAT for liver disease associated with Alpha-1 antitrypsin deficiency.

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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