December 14, 2015

Arrowhead Reports Fiscal 2015 Year End Results

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

PASADENA, Calif.--(BUSINESS WIRE)-- Arrowhead Research Corporation (NASDAQ: ARWR) today announced financial results for its fiscal 2015 fourth quarter and year ended September 30, 2015. The company is hosting a conference call at 4:30 p.m. EST to discuss results.

Conference Call and Webcast Details

Investors may access a live audio webcast on the Company's website at http://ir.arrowheadresearch.com/events.cfm. For analysts that wish to participate in the conference call, please dial 855-215-6159 or 315-625-6887 and enter Conference ID 99535530.

A replay of the webcast will be available on the company's website 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 3 days. To access the audio replay, dial 404-537-3406 and enter Conference ID 99535530.

Fiscal 2015 Fourth Quarter and Recent Company Highlights

ARC-520

- Presented data at AASLD Liver Meeting 2014 showing statistically significant reduction in HBsAg through day 43 after a single injection ($p < 0.05$) in human clinical trials
- Submitted an Investigational New Drug (IND) application to the U.S. Food and Drug Administration and submitted additional clinical trial authorization applications with regulatory authorities in various jurisdictions in Europe, Asia, and Australia/New Zealand for ARC-520
- Initiated dosing in Heparc-2004, a multiple-dose Phase 2b clinical study of ARC-520 in the U.S.
- Initiated multiple-dose Heparc-2002 and Heparc-2003 Phase 2b studies of ARC-520 in Europe and Asia
- Hosted an analyst day to discuss top-line findings from the Heparc-2001 Phase 2a clinical study of ARC-520 and findings from a study of 9 chimpanzees that have been treated monthly with ARC-520 for between 6 and 11 months. Key messages included the following:
  - Arrowhead's proprietary DPC™ platform can effectively and consistently knock down target genes in humans
  - ARC-520 achieved significant HBV s-Antigen (HBsAg) reductions in humans, particularly in treatment naïve, HBeAg-positive patients
  - Arrowhead identified a large target HBV population for ARC-520 and described a new paradigm for the HBV lifecycle
  - ARC-520 induced deep HBsAg reduction in chronically HBV infected chimpanzees
  - ARC-520 was well tolerated, no serious or severe adverse events were reported in these studies
  - Arrowhead expanded its HBV portfolio by nominating ARC-521, an additional clinical candidate that is complementary to ARC-520
- Presented data at the AASLD Liver Meeting 2015 including the following:
  - ARC-520 led to robust, sustained anti-viral effects in chimpanzees with chronic HBV, and we also described an important new discovery that HBV DNA integrated into the host genome is likely an important source of HBV surface antigen (HBsAg) production
  - In a Phase 2a clinical study, ARC-520 effectively reduced HBV viral antigens derived from cccDNA. HBV surface antigen (HBsAg) was reduced substantially with a maximum reduction of 1.9 logs (99%) and a mean

maximum reduction of 1.5 logs (96.8%) in treatment naïve e-antigen (HBeAg)-positive patients

- Presented data at Hep DART 2015 showing that ARC-520 led to immune reactivation in 7 of 9 chimpanzees with chronic hepatitis B infection

ARC-AAT

- Presented data at AASLD Liver Meeting 2014
  - Repeat dosing of ARC-AAT in primates showed reduction of approximately 90% of serum alpha-1 antitrypsin (AAT) with long duration of effect suggesting that monthly or less frequent dosing may be sufficient for sustained suppression of hepatic AAT production
  - ARC-AAT abstract highlighted in the AASLD President's Press Conference as a promising new treatment
- Filed for regulatory approval to begin a Phase 1 clinical trial of ARC-AAT for the treatment of liver disease associated with alpha-1 antitrypsin deficiency
- Initiated dosing in a Phase 1 clinical trial of ARC-AAT
- Completed dosing of Part A of the ARC-AAT phase 1 study in healthy volunteers, and transitioned the study into Part B in patients with PiZZ genotype alpha-1 antitrypsin deficiency
- Received Orphan Drug Designation from the United States Food and Drug Administration
- Expanded Part B of the Phase 1 study of ARC-AAT to include additional treatment sites in Europe, Australia, and New Zealand

Platform and Early Pipeline

- Acquired Novartis Institutes for BioMedical Research, Inc ("Novartis") entire RNAi research and development portfolio and associated assets, including:
  - Multiple patent families covering RNAi-trigger design rules and modifications that fall outside of key patents controlled by competitors, which the Company believes provides freedom to operate for any target and indication
  - Novel intracellular targeting ligands that enhance the activity of RNAi-triggers by targeting the RNA-induced silencing complex (RISC) more effectively and improving stability once RISC is loaded
  - An assignment of Novartis' license from Alnylam Pharmaceuticals, Inc. ("Alnylam") granting Arrowhead access to Alnylam intellectual property, excluding delivery, for 30 gene targets chosen by Novartis
  - A pipeline of three candidates initiated by Novartis for which Novartis has developed varying amounts of preclinical data
- Published new data in the *Journal of Controlled Release*, 209 (2015) 57-66, on a subcutaneously administered formulation of its DPC™ delivery system
- Presented data at the TIDES Conference on the development of ARC-F12, an RNAi therapeutic for factor 12 mediated hereditary angioedema and thromboembolic diseases
- Nominated ARC-HIF2 against clear cell renal cell carcinoma as Arrowhead's first therapeutic candidate delivered using a new DPC™ designed to target tissues outside of the liver
- Presented data at the Annual Meeting of the Oligonucleotide Therapeutics Society on the development of ARC-LPA against cardiovascular disease, which uses a new subcutaneous delivery construct that Arrowhead has developed

Selected Fiscal 2015 Year End Financial Results

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ARROWHEAD RESEARCH CORPORATION
CONSOLIDATED CONDENSED FINANCIAL INFORMATION

	Â	Year Ended September 30,		Â
OPERATING SUMMARY		**2015**	**2014**	
REVENUE		**$ 382,000**	**$ 175,000**	
OPERATING EXPENSES				
Research and development		47,267,361	23,138,050	

Acquired in-process research and development	10,142,786	-
Salaries and payroll-related costs	16,554,008	12,829,355
General and administrative expenses	7,931,184	5,894,008
Stock-based compensation	10,232,897	5,696,173
Depreciation and amortization	2,336,207	1,345,655
Impairment expense	-	2,172,387
Contingent consideration - fair value adjustments	Â 1,891,533	Â 2,375,658
TOTAL OPERATING EXPENSES	Â 96,355,976	Â 53,451,286
OPERATING LOSS	**(95,973,976)**	**(53,276,286)**
OTHER INCOME/(EXPENSE), LOSS FROM DISCONTINUED OPERATIONS, PROVISION FOR INCOME TAXES	Â **4,033,094**	Â **(5,449,126)**
NET LOSS	**$ (91,940,882)**	**$ (58,725,412)**
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EARNINGS PER SHARE (BASIC AND DILUTED):	**$ (1.60)**	**$ (1.25)**
WEIGHTED AVERAGE SHARES OUTSTANDING	Â **57,358,442**	Â **46,933,030**
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FINANCIAL POSITION SUMMARY	**September 30,**	
	2015	**2014**
CASH AND CASH EQUIVALENTS	**81,214,354**	**132,510,610**
SHORT AND LONG-TERM INVESTMENTS	Â **17,539,902**	Â **44,741,378**
TOTAL CASH RESOURCES (CASH, CASH EQUIVALENTS AND INVESTMENTS)	**98,754,256**	**177,251,988**
OTHER ASSETS	Â **33,513,658**	Â **5,564,768**
TOTAL ASSETS	Â **132,267,914**	Â **182,816,756**
TOTAL LIABILITIES	**22,646,280**	**16,831,501**
TOTAL STOCKHOLDERS' EQUITY	Â **109,621,634**	Â **165,985,255**
TOTAL LIABILITIES AND STOCKHOLDERS' EQUITY	Â **132,267,914**	Â **182,816,756**
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SHARES OUTSTANDING	**59,544,677**	**54,656,936**
PROFORMA SHARES OUTSTANDING (INCLUDING CONVERSION OF PREFERRED SHARES)	**62,215,667**	**58,644,142**
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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. The mean estimated prevalence of AATD in the U.S. is 1 per 3000-5000, or approximately 100,000 patients. AATD is also an important cause of pediatric liver disease with an estimated prevalence in children of approximately 20,000 patients, and 50-80% likely to manifest liver disease during childhood. It is a rare disease that appears to be frequently misdiagnosed or undiagnosed. ARC-AAT, which was granted orphan drug designation, 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 is believed to be 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. Arrowhead is conducting a single dose Phase 1 clinical study of ARC-AAT, with part A in healthy volunteers and part B in AATD patients.

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 is conducting Phase 2b multiple dose and combination 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 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 and ARC-521 for chronic hepatitis B virus, ARC-AAT for liver disease associated with alpha-1 antitrypsin deficiency, ARC-F12 for hereditary angioedema and thromboembolic diseases, and ARC-HIF2 for renal cell carcinoma.

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.

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