



February 9, 2016

## Arrowhead Reports Fiscal 2016 First Quarter 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 2016 first quarter ended December 31, 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 40876476.

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 40876476.

### Fiscal 2016 First Quarter and Recent Company Highlights

#### 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. 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
- | Began dosing in the Phase 2b MONARCH combination study

#### ARC-AAT

- | Expanded Part A of the Phase 1 study to test additional dose levels in healthy volunteers and expanded Part B to add additional treatment sites for patients with alpha-1 antitrypsin deficiency
- | Received Orphan Drug Designation by the European Medicines Agency

### Platform and Early Pipeline

- | 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 2016 First Quarter Financial Results

**ARROWHEAD RESEARCH CORPORATION**  
**CONSOLIDATED CONDENSED FINANCIAL INFORMATION (unaudited)**

<b><u>OPERATING SUMMARY</u></b>	<b>Three Months Ended December 31,</b>	
	<b>2015</b>	<b>2014</b>
<b>REVENUE</b>	<b>\$ 43,750</b>	<b>\$ 170,750</b>
<b>OPERATING EXPENSES</b>		
Research and development	10,338,833	17,744,312
Salaries and payroll-related costs	3,919,886	3,150,617
General and administrative expenses	1,951,609	2,086,202
Stock-based compensation	2,380,343	2,014,856
Depreciation and amortization	794,349	290,039
<b>TOTAL OPERATING EXPENSES</b>	<b>19,385,020</b>	<b>25,286,026</b>
<b>OPERATING LOSS</b>	<b>(19,341,270)</b>	<b>(25,115,276)</b>
<b>OTHER INCOME/(EXPENSE), PROVISION FOR INCOME TAXES</b>	<b>76,856</b>	<b>2,539,994</b>
<b>NET LOSS</b>	<b>\$ (19,264,414)</b>	<b>\$ (22,575,282)</b>
<b>EARNINGS PER SHARE (BASIC AND DILUTED):</b>	<b>\$ (0.32)</b>	<b>\$ (0.41)</b>
<b>WEIGHTED AVERAGE SHARES OUTSTANDING</b>	<b>59,548,672</b>	<b>54,692,392</b>
<b><u>FINANCIAL POSITION SUMMARY</u></b>	<b>December 31,</b>	<b>September 30,</b>
	<b>2015</b>	<b>2015</b>
<b>CASH AND CASH EQUIVALENTS</b>	<b>62,165,088</b>	<b>81,214,354</b>
<b>SHORT-TERM INVESTMENTS</b>	<b>14,431,498</b>	<b>17,539,902</b>
<b>TOTAL CASH RESOURCES (CASH, CASH EQUIVALENTS AND INVESTMENTS)</b>	<b>76,596,586</b>	<b>98,754,256</b>
<b>OTHER ASSETS</b>	<b>34,784,553</b>	<b>33,513,658</b>
<b>TOTAL ASSETS</b>	<b>111,381,139</b>	<b>132,267,914</b>
<b>TOTAL LIABILITIES</b>	<b>19,012,747</b>	<b>22,646,280</b>
<b>TOTAL STOCKHOLDERS' EQUITY</b>	<b>92,368,392</b>	<b>109,621,634</b>
<b>TOTAL LIABILITIES AND STOCKHOLDERS' EQUITY</b>	<b>111,381,139</b>	<b>132,267,914</b>
<b>SHARES OUTSTANDING</b>	<b>59,627,499</b>	<b>59,544,677</b>
<b>PROFORMA SHARES OUTSTANDING (INCLUDING CONVERSION OF PREFERRED SHARES)</b>	<b>62,298,489</b>	<b>62,215,667</b>

#### **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 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. ARC-AAT was granted orphan drug designation in both the United States and in Europe, the latter being held on Arrowhead's behalf by a local EU representative Pharma Gateway AB. Arrowhead is conducting a 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 seroconversion. 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. Arrowhead is currently conducting Phase 2b multiple dose and combination studies in chronic HBV patients. In clinical studies to date, the most common reported adverse events in all subjects completing treatment were upper

respiratory infection and headache.

## 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](https://twitter.com/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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