



May 10, 2016

## Arrowhead Reports Fiscal 2016 Second Quarter Results

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

PASADENA, Calif.--(BUSINESS WIRE)-- Arrowhead Pharmaceuticals Inc. (NASDAQ: ARWR) today announced financial results for its fiscal 2016 second quarter ended March 31, 2016. The company is hosting a conference call at 4:30 p.m. EDT to discuss results.

### Conference Call and Webcast Details

Investors may access a live audio webcast on the Company's website at <http://ir.arrowheadpharma.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 5054757.

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 5054757.

### Fiscal 2016 Second Quarter and Recent Company Highlights

#### Corporate Events

- Completed corporate name change to Arrowhead Pharmaceuticals, Inc. to better reflect the company's stage of development and launched a new website at [ArrowheadPharma.com](http://ArrowheadPharma.com) with an updated corporate identity

#### ARC-520

- Began dosing patients in three Phase 2b studies: the MONARCH study, 2007 long-term extension, and 2001 open-label extension
- Presented promising ARC-520 hepatitis B data at The International Liver Congress™ 2016, including the following key findings:
  - ARC-520 and entecavir produced rapid HBV DNA suppression with all hepatitis B e-antigen (HBeAg) positive, treatment naïve patients achieving serum HBV DNA reductions of up to 5.5 log (99.9997%), and all HBeAg negative, treatment naïve patients achieving reductions that put them below the limit of quantitation
  - ARC-520 effectively inhibited HBV cccDNA-derived mRNA with observed viral protein reduction in HBV patients of up to 2.0 log (99%) after a single dose
  - ARC-520 had a long duration of effect with HBsAg still reduced by 83% after 2 months and 75% after 3 months, which is the final time point of the study, after a single dose
  - Based on HBsAg epitope profile analysis, poster authors and Arrowhead collaborators had previously identified a predictive hepatitis B surface-antigen (HBsAg) Clearance Profile associated with HBsAg clearance in antiviral therapy cohorts
  - There was a significant association between the development of an HBsAg Clearance Profile and ARC-520 therapy in HBV patients
  - Complexed HBsAg antibodies (anti-HBs) were developed and detected in HBV patients treated with ARC-520, which may represent a recovery of the immune system response
  - After monthly administration of 6-11 doses of ARC-520 in chimpanzees chronically infected with HBV, the ARC-520 target site sequences remained virtually unchanged, indicating that no drug resistance developed during the treatment period

#### ARC-521

- Filed for regulatory clearance to begin a Phase 1/2 first-in-human study to assess single and multiple-doses of ARC-

521 in healthy volunteers and HBV patients

## ARC-AAT

- ┆ Received Orphan Drug Designation by the European Medicines Agency

## Platform and Early Pipeline

- ┆ Presented promising new preclinical data the 2016 American Academy of Allergy, Asthma & Immunology Annual Meeting suggesting that ARC-F12, an RNAi therapeutic that inhibits the production of Factor XII (F12), has the potential to treat hereditary angioedema and to prevent thrombosis
- ┆ Presented data at the American Association for Cancer Research Annual Meeting 2016 (AACR16), showing that ARC-HIF2 inhibited tumor growth and promoted tumor cell death and structural degeneration in two different renal cell carcinoma tumor bearing mouse models
  - ┆ These data also show that important advancements are being made to Arrowhead's Dynamic Polyconjugate™ (DPC™) delivery platform to include extra-hepatic targeting capabilities
- ┆ Presented data on ARC-LPA, a preclinical development program targeting lipoprotein (a), or Lp(a), for the treatment of cardiovascular disease, at the Arteriosclerosis, Thrombosis and Vascular Biology | Peripheral Vascular Disease (ATVBJPVD) 2016 Scientific Sessions
  - ┆ These data show that ARC-LPA and Arrowhead's new delivery vehicles designed for subcutaneous administration can induce deep target gene knockdown with long duration of effect that may enable monthly, bi-monthly, or even less frequent administration

## Selected Fiscal 2016 Second Quarter Financial Results

### ARROWHEAD PHARMACEUTICALS, INC. CONSOLIDATED CONDENSED FINANCIAL INFORMATION (unaudited)

<u>OPERATING SUMMARY</u>	Three Months Ended March 31,		Six Months Ended March 31,	
	2016	2015	2016	2015
<b>REVENUE</b>	\$ 43,750	\$ 43,750	\$ 87,500	\$ 214,500
<b>OPERATING EXPENSES</b>				
Research and development	10,020,826	11,640,794	20,359,659	29,387,524
Acquired in-process research and development	-	10,142,786	-	10,142,786
Salaries and payroll-related costs	4,248,693	3,541,652	8,168,579	6,692,268
General and administrative expenses	3,818,335	1,696,623	5,769,944	3,782,826
Stock-based compensation	2,416,839	2,205,079	4,797,182	4,219,935
Depreciation and amortization	803,912	449,559	1,598,261	739,598
<b>TOTAL OPERATING EXPENSES</b>	<u>21,308,605</u>	<u>29,676,493</u>	<u>40,693,625</u>	<u>54,964,937</u>
<b>OPERATING LOSS</b>	<b><u>(21,264,855)</u></b>	<b><u>(29,632,743)</u></b>	<b><u>(40,606,125)</u></b>	<b><u>(54,750,437)</u></b>
<b>OTHER INCOME/(EXPENSE), PROVISION FOR INCOME TAXES</b>	<u>448,995</u>	<u>948,750</u>	<u>525,851</u>	<u>3,488,743</u>
<b>NET LOSS</b>	<b><u>\$(20,815,860)</u></b>	<b><u>\$(28,683,993)</u></b>	<b><u>\$(40,080,274)</u></b>	<b><u>\$ (51,261,694)</u></b>
<b>EARNINGS PER SHARE (BASIC AND DILUTED):</b>	<b><u>\$ (0.35)</u></b>	<b><u>\$ (0.51)</u></b>	<b><u>\$ (0.67)</u></b>	<b><u>\$ (0.93)</u></b>
<b>WEIGHTED AVERAGE SHARES OUTSTANDING</b>	<b><u>59,779,128</u></b>	<b><u>55,719,923</u></b>	<b><u>59,663,270</u></b>	<b><u>55,200,512</u></b>
<b><u>FINANCIAL POSITION SUMMARY</u></b>			<b>March 31,</b>	<b>September 30,</b>
			<b>2016</b>	<b>2015</b>
<b>CASH AND CASH EQUIVALENTS</b>			<u>50,300,847</u>	<u>81,214,354</u>
<b>SHORT-TERM INVESTMENTS</b>			<u>11,160,442</u>	<u>17,539,902</u>
<b>TOTAL CASH RESOURCES (CASH, CASH EQUIVALENTS AND INVESTMENTS)</b>			<u>61,461,289</u>	<u>98,754,256</u>
<b>OTHER ASSETS</b>			<u>33,146,182</u>	<u>33,513,658</u>
<b>TOTAL ASSETS</b>			<u>94,607,471</u>	<u>132,267,914</u>
<b>TOTAL LIABILITIES</b>			<u>20,681,234</u>	<u>22,646,280</u>

TOTAL STOCKHOLDERS' EQUITY	<u>73,926,237</u>	<u>109,621,634</u>
TOTAL LIABILITIES AND STOCKHOLDERS' EQUITY	<u>94,607,471</u>	<u>132,267,914</u>
SHARES OUTSTANDING	59,960,711	59,544,677
PROFORMA SHARES OUTSTANDING (INCLUDING CONVERSION OF PREFERRED SHARES)	62,631,701	62,215,667

### About Arrowhead Pharmaceuticals

Arrowhead Pharmaceuticals develops medicines that treat intractable diseases by silencing the genes that cause them. Using a broad portfolio of RNA chemistries and efficient modes of delivery, Arrowhead therapies trigger the RNA interference mechanism to induce rapid, deep, and durable knockdown of target genes. RNA interference, or RNAi, is a mechanism present in living cells that inhibits the expression of a specific gene, thereby affecting the production of a specific protein. Arrowhead's RNAi-based therapeutics leverage this natural pathway of gene silencing. The company's pipeline includes ARC-520 and ARC-521 for chronic hepatitis B virus infection, ARC-AAT for liver disease associated with alpha-1 antitrypsin deficiency, ARC-F12 for hereditary angioedema and thromboembolic disorders, ARC-LPA for cardiovascular disease, and ARC-HIF2 for renal cell carcinoma.

For more information please visit [www.arrowheadpharma.com](http://www.arrowheadpharma.com), or follow us on Twitter [@ArrowheadPharma](https://twitter.com/ArrowheadPharma). To be added to the Company's email list and receive news directly, please visit <http://ir.arrowheadpharma.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. Our 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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Source: Arrowhead Pharmaceuticals, Inc.

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