December 14, 2016

Arrowhead Reports Fiscal 2016 Year End Results

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

PASADENA, Calif.--(BUSINESS WIRE)-- Arrowhead Pharmaceuticals, Inc. (NASDAQ: ARWR) today announced financial results for its fiscal 2016 fourth quarter and year ended September 30, 2016. 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.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 33791749.

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 33791749.

Selected Fiscal 2016 and Recent Events

- Discontinued development of ARC-520, ARC-521, and ARC-AAT in November 2016
  - The Company announced that it would be discontinuing these clinical programs, which utilized the intravenously administered DPC_{iv}^{TM}, or EX1, delivery vehicle, and redeploying its resources and focus toward utilizing the Company's new proprietary subcutaneous and extra-hepatic delivery systems
  - The decision to discontinue development of EX1-containing programs was based primarily on two factors:
    - During ongoing discussions with regulatory agencies and outside experts, it became apparent that there would be substantial delays in all clinical programs that utilize EX1, while the Company further explored the cause of deaths in a non-clinical toxicology study in non-human primates exploring doses of EX1 higher than those planned to be used in humans
    - The Company has made substantial advances in RNA chemistry and targeting resulting in large potency gains for subcutaneous administered and extra-hepatic RNAi-based development programs
  - Because of the discontinuation of its existing clinical programs, the Company also reduced its workforce by approximately 30%, while maintaining resources necessary to support current and potential partner-based programs and the Company's pipeline
- Entered into two collaboration and license agreements with Amgen
  - Total deal value of up to $673.5 million
  - Arrowhead received $56.5 million upfront:
    - $35 million in upfront cash payments, $21.5 million equity investment
  - Up to low double-digit royalties for ARO-LPA and single-digit royalties for the undisclosed target, ARO- AMG1
  - Amgen receives:
    - Exclusive license to ARO-LPA program
    - Option for an additional candidate against an undisclosed target, ARO- AMG1
  - Amgen will be wholly responsible for funding and conducting all clinical development and commercialization
- Continued progress on preclinical candidates including ARO-HBV, ARO-AAT, ARO-F12, ARO-LPA, and ARO-HIF2
  - Regarding ARO-F12 and ARO-LPA:
    - Presented preclinical data at the American Heart Association's Scientific Sessions 2016 for two development programs using Arrowhead's proprietary subcutaneous delivery platform:
    - RNAi triggers against Factor 12 (F12) showed dose dependent reductions in serum F12
    - A statistically significant reduction (p=0.002) in thrombus weight was observed at greater than 95% F12

knockdown in a rat arterio-venous shunt model

- There was no increased bleeding risk in ARO- F12-treated mice, even with greater than 99% knockdown of F12 levels
- RNAi triggers against Lipoprotein (a) [Lp(a)] led to greater than 98% maximum knockdown after a single 3 mg/kg SQ dose in Transgenic mice
- In an atherosclerosis model, data suggest that RNAi triggers can be effectively delivered to a fatty liver using the subcutaneous delivery platform

- Regarding ARO-HIF2
  - Presented preclinical data showing that ARO-HIF2 inhibited renal cell carcinoma growth and promoted tumor cell death in its preclinical studies

- Strengthened the Company's balance sheet with August 2016 private offering and Amgen agreement upfront payments
  - In August 2016, the Company sold 7.6 million shares of Common Stock to certain institutional investors and received net proceeds of approximately $43.2 million
  - As part of the collaboration and license agreements as well as a Common Stock Purchase Agreement with Amgen, $14 million of the total $56.5 million upfront cash payments and equity investments were received in September 2016, and the remaining $42.5 million was received in November 2016
- Continued progress on former drug candidates prior to the discontinuations
  - Presented preclinical and clinical data on former drug candidate ARC-AAT at the Liver Meeting
  - In a first-in-human clinical study, ARC-AAT was well tolerated and induced deep and durable reduction of the target AAT protein
  - The preclinical data suggest a possible improvement of liver health and arrest of further damage from treatment with ARC-AAT
  - Advanced former drug candidate ARC-521 into a Phase 1/2 study
  - Conducted multiple dose and combination studies of former drug candidate ARC-520

Selected Fiscal 2016 Year End Financial Results

ARROWHEAD PHARMACEUTICALS, INC.
CONSOLIDATED FINANCIAL INFORMATION

	Year Ended September 30	
OPERATING SUMMARY	**2016**	**2015**
REVENUE	**$ 158,333**	**$ 382,000**
OPERATING EXPENSES		
Research and development	41,454,452	47,267,361
Acquired in-process research and development	-	10,142,786
Salaries and payroll-related costs	19,461,656	16,554,008
General and administrative expenses	9,940,737	7,931,184
Stock-based compensation	11,595,816	10,232,897
Depreciation and amortization	3,260,045	2,336,207
Impairment expense	2,050,817	-
Contingent consideration - fair value adjustments	(5,862,464)	1,891,533
TOTAL OPERATING EXPENSES	81,901,059	96,355,976
OPERATING LOSS	**(81,742,726)**	**(95,973,976)**
OTHER INCOME/(EXPENSE), PROVISION FOR INCOME TAXES	**19,724**	**4,033,094**
NET LOSS	**$ (81,723,002)**	**$ (91,940,882)**
EARNINGS PER SHARE (BASIC AND DILUTED):	**$ (1.34)**	**$ (1.60)**
WEIGHTED AVERAGE SHARES OUTSTANDING	**61,050,880**	**57,358,442**

FINANCIAL POSITION SUMMARY	**September 30,**

	2016	2015
CASH AND CASH EQUIVALENTS	**85,366,448**	**81,214,354**
SHORT-TERM INVESTMENTS	**-**	**17,539,902**
TOTAL CASH RESOURCES (CASH, CASH EQUIVALENTS AND INVESTMENTS)	**85,366,448**	**98,754,256**
OTHER ASSETS	**42,810,057**	**33,513,658**
TOTAL ASSETS	**128,176,505**	**132,267,914**
TOTAL LIABILITIES	**33,152,246**	**22,646,280**
TOTAL STOCKHOLDERS' EQUITY	**95,024,259**	**109,621,634**
TOTAL LIABILITIES AND STOCKHOLDERS' EQUITY	**128,176,505**	**132,267,914**
SHARES OUTSTANDING	**69,746,685**	**59,544,677**
PROFORMA SHARES OUTSTANDING (INCLUDING CONVERSION OF PREFERRED SHARES)	**72,417,675**	**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.

For more information, please visit www.arrowheadpharma.com, or follow us on Twitter @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 the safety and efficacy of our product candidates, the duration and impact of regulatory delays in our clinical programs, 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.

Source: Arrowhead Pharmaceuticals, Inc.

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