



Arrowhead Pharmaceuticals Reports Fiscal 2018 Year End Results

December 11, 2018

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

PASADENA, Calif.--(BUSINESS WIRE)--Dec. 11, 2018-- Arrowhead Pharmaceuticals Inc. (NASDAQ: ARWR) today announced financial results for its fiscal 2018 fourth quarter and year ended September 30, 2018. 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 provide Conference ID 6744427.

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 855-859-2056 or 404-537-3406 and provide Conference ID 6744427.

Selected Fiscal 2018 and Recent Events

- Hosted an Analyst R&D Day in September 2017 to highlight the following:
 - The Targeted RNAi Molecule platform, or TRiM™, which utilizes ligand-mediated delivery and is designed to enable tissue-specific targeting, while being structurally simple. The TRiM™ platform offers several potential competitive advantages including:
 - A more sophisticated RNAi trigger selection and screening process that identifies potent sequences rapidly in locations that RNAi competitors may miss
 - Multiple routes of administration including subcutaneous, intravenous, and inhaled
 - Faster time to clinical candidates
 - Optimal pharmacologic activity and long duration-of-effect
 - Potentially wide safety margins
 - Simplified manufacturing at reduced cost
 - And, the promise of taking RNAi to tissues beyond the liver
 - Presented new clinical data at HEP DART 2017 and EASL 2018 demonstrating up to 5.3 Log10 reduction in HBV s-antigen and a Sustained Host Response in 50% of hepatitis B patients following first generation RNAi therapy, ARC-520, in the 2001 open label extension study
 - One patient serocleared all viral markers, including HBsAg
 - Began a Phase 1 study of ARO-AAT, Arrowhead's second generation subcutaneously administered RNAi therapeutic being developed as a treatment for a rare genetic liver disease associated with alpha-1 antitrypsin deficiency
 - Began a Phase 1/2 study of ARO-HBV, a third-generation subcutaneously administered RNAi therapeutic candidate being developed as a potential treatment for patients with chronic hepatitis B virus infection
 - Announced that Amgen had administered the first dose of AMG 890, formerly ARO-LPA, in a Phase 1 clinical study, which earned Arrowhead a \$10 million milestone payment
 - Presented clinical data on ARO-AAT at the Alpha-1 National Education Conference and at the AASLD Liver Meeting 2018 demonstrating:
 - Three monthly doses of 300 mg ARO-AAT led to reductions in serum alpha-1 antitrypsin to below the level of quantitation in 100% of subjects
 - Reductions were sustained for greater than 14 weeks indicating that quarterly or less frequent dosing appears feasible
 - Single and multiple doses of ARO-AAT appeared to be well-tolerated at all doses tested
 - Presented clinical data on ARO-HBV at the World Gastroenterologists Summit and at the AASLD Liver Meeting 2018 demonstrating:
 - Mean HBsAg reduction of -1.9 Log10 (-98.7%) with a range of -1.3 Log10 (-95.0%) to -3.8 Log10 (-99.98%)

- ARO-HBV appeared to be well-tolerated at monthly doses up to 400 mg
- Signed a license agreement with Janssen Pharmaceuticals, Inc., part of the Janssen Pharmaceuticals Companies of Johnson & Johnson, for ARO-HBV and a collaboration agreement for up to three RNAi therapeutic candidates that use our proprietary TRiM™ platform against new targets to be selected by Janssen
 - The total potential deal value is approximately \$3.7 billion plus royalties on commercial sales
 - Received \$175 million as an upfront payment and received \$75 million in the form of an equity investment by Johnson & Johnson Innovation – JJDC, Inc., at a price of \$23.00 per share of Arrowhead common stock
- Hosted an R&D Day in October 2018 to discuss in more detail our emerging pipeline of RNAi therapeutics that leverage the TRiM™ platform
- Filed for regulatory clearance to begin a Phase 1 study of ARO-ANG3, an RNAi-based investigational medicine targeting angiotensin like protein 3 (ANGPTL3) being developed for the treatment of dyslipidemias and metabolic diseases

Selected Fiscal 2018 Year End Financial Results

ARROWHEAD PHARMACEUTICALS, INC. CONSOLIDATED FINANCIAL INFORMATION

<u>OPERATING SUMMARY</u>	<u>Year Ended September 30,</u>	
	<u>2018</u>	<u>2017</u>
REVENUE	\$ 16,142,321	\$ 31,407,709
OPERATING EXPENSES		
Research and development	52,968,505	50,904,466
General and administrative expenses	19,110,051	17,499,152
TOTAL OPERATING EXPENSES	<u>72,078,556</u>	<u>68,403,618</u>
OPERATING LOSS	(55,936,235)	(36,995,909)
OTHER INCOME/(EXPENSE), PROVISION FOR INCOME TAXES	1,485,757	2,615,614
NET LOSS	\$ (54,450,478)	\$ (34,380,295)
NET LOSS PER SHARE (BASIC AND DILUTED):	\$ (0.65)	\$ (0.47)
WEIGHTED AVERAGE SHARES OUTSTANDING	83,638,469	73,898,598
<u>FINANCIAL POSITION SUMMARY</u>	<u>September 30,</u>	<u>September 30,</u>
	<u>2018</u>	<u>2017</u>
CASH AND CASH EQUIVALENTS	\$ 30,133,213	\$ 24,838,567
SHORT-TERM INVESTMENTS	46,400,176	40,769,539
TOTAL CASH RESOURCES (CASH AND INVESTMENTS)	76,533,389	65,608,106
OTHER ASSETS	35,076,562	38,414,174
TOTAL ASSETS	111,609,951	104,022,280
TOTAL LIABILITIES	16,368,950	23,155,118
TOTAL STOCKHOLDERS' EQUITY	95,241,001	80,867,162
TOTAL LIABILITIES AND STOCKHOLDERS' EQUITY	\$ 111,609,951	\$ 104,022,280
SHARES OUTSTANDING	88,505,302	74,785,426

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](https://twitter.com/ArrowheadPharma). To be added to the Company's email list and receive news directly, please visit <http://ir.arrowheadpharma.com/email-alerts>.

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 likelihood and timing of the receipt of future milestone and licensing fees, the future success of our scientific studies, our ability to successfully develop and commercialize 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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