Arrowhead Pharmaceuticals Reports Fiscal 2019 Third Quarter Results

August 5, 2019

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

PASADENA, Calif.--(BUSINESS WIRE)--Aug. 5, 2019-- Arrowhead Pharmaceuticals Inc. (NASDAQ: ARWR) today announced financial results for its fiscal 2019 third quarter ended June 30, 2019. 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 provide Conference ID 3249189.

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 3249189.

Selected Fiscal 2019 Third Quarter and Recent Events

- Received U.S. Food and Drug Administration (FDA) clearance to begin an adaptive design Phase 2/3 trial, called SEQUOIA, with the potential to serve as a pivotal registrational 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
- Secured first regulatory clearance in the United Kingdom for the ARO-AAT 2002 study, a pilot open-label, multi-dose, Phase 2 study to assess changes in a novel histological activity scale in response to ARO-AAT over time in patients with alpha-1 antitrypsin deficiency associated liver disease
- Expanded the AROHBV1001 Phase 1/2 study to include a new triple combination cohort that includes: JNJ-3989, formerly ARO-HBV; JNJ-6379, Janssen's investigational orally administered capsid assembly modulator of the class that forms normal capsid structures; and, a nucleos(t)ide analog, or NUC
    - In connection with the start of dosing of this cohort, Arrowhead earned a $25 million milestone payment from Janssen
- Received orphan drug designation from FDA for ARO-APOC3 for the treatment of familial chylomicronemia syndrome
- Received FDA Fast Track designation for ARO-AAT
    - Fast Track is a process designed to facilitate the development and expedite the review of drugs to treat serious conditions and fill an unmet medical need. The purpose is to get important new drugs to the patient earlier
- Received orphan drug designation from FDA for ARO-ANG3 for the treatment of homozygous familial hypercholesterolemia
- Completed discovery and development work on ARO-HSD, a previously undisclosed liver-targeted candidate targeting HSD17B13, a hydroxysteroid dehydrogenase involved in the metabolism of hormones, fatty acids and bile acids, that is now in IND-enabling GLP-toxicology studies, and, pending success in the tox program, on schedule for a CTA filing at the end of 2019
- Started IND-enabling GLP-toxicology studies for ARO-HIF2, designed to inhibit the production of HIF-2a for the treatment of clear cell renal cell carcinoma, to support, pending success in the tox program, a CTA filing at the end of 2019
- Completed dosing in the single-ascending dose portions of the Phase 1 studies of Arrowhead's two wholly-owned cardiometabolic candidates, ARO-APOC3 and ARO-ANG3, and progressed towards the multiple-dose portions of the Phase 1 studies in various patient populations

Selected Fiscal 2019 Third Quarter Financial Results

ARROWHEAD PHARMACEUTICALS, INC.

CONSOLIDATED CONDENSED FINANCIAL INFORMATION (unaudited)

	Three Months Ended June 30,	Nine Months Ended June 30,

OPERATING SUMMARY	2019	2018	2019	2018
REVENUE	**$ 42,696,636**	**$ 727,375**	**$ 125,502,807**	**$ 4,887,321**
OPERATING EXPENSES				
Research and development	19,291,710	12,052,653	57,662,381	36,974,675
General and administrative expenses	4,809,177	4,594,441	16,287,841	12,679,822
TOTAL OPERATING EXPENSES	24,100,887	16,647,094	73,950,222	49,654,497
OPERATING INCOME (LOSS)	**18,595,749**	**(15,919,719)**	**51,552,585**	**(44,767,176)**
OTHER INCOME/(EXPENSE), PROVISION FOR INCOME TAXES	**1,739,959**	**313,702**	**4,717,359**	**1,077,919**
NET INCOME (LOSS)	**$ 20,335,708**	**$ (15,606,017)**	**$ 56,269,944**	**$ (43,689,257)**
NET INCOME (LOSS) PER SHARE (DILUTED)	**$ 0.21**	**$ (0.18)**	**$ 0.58**	**$ (0.53)**
WEIGHTED AVERAGE SHARES OUTSTANDING (DILUTED)	**98,884,744**	**87,634,435**	**97,814,019**	**82,149,381**

FINANCIAL POSITION SUMMARY	June 30, 2019	September 30, 2018
CASH AND CASH EQUIVALENTS	**$ 188,331,747**	**$ 30,133,213**
RESTRICTED CASH	**1,000,000**	**-**
SHORT-TERM INVESTMENTS	**48,567,215**	**46,400,176**
LONG-TERM INVESTMENTS	**57,555,499**	**-**
TOTAL CASH RESOURCES (CASH AND INVESTMENTS)	**295,454,461**	**76,533,389**
OTHER ASSETS	**41,373,984**	**35,076,562**
TOTAL ASSETS	**336,828,445**	**111,609,951**
TOTAL CURRENT DEFERRED REVENUE	**78,681,868**	**600**
TOTAL LONG TERM DEFERRED REVENUE	**22,258,994**	**-**
OTHER LIABILITIES	**10,217,543**	**16,368,350**
TOTAL LIABILITIES	**111,158,405**	**16,368,950**

TOTAL STOCKHOLDERS' EQUITY	**225,670,040**	**95,241,001**
TOTAL LIABILITIES AND STOCKHOLDERS' EQUITY	**$ 336,828,445**	**$ 111,609,951**
SHARES OUTSTANDING	**95,207,637**	**88,502,302**

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/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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