Arrowhead Pharmaceuticals Reports Fiscal 2019 Second Quarter Results

May 8, 2019

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

PASADENA, Calif.--(BUSINESS WIRE)--May 8, 2019-- Arrowhead Pharmaceuticals Inc. (NASDAQ: ARWR) today announced financial results for its fiscal 2019 second quarter ended March 31, 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 5049067.

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 5049067.

Selected Fiscal 2019 Second Quarter and Recent Events

- Began dosing in a Phase 1 single and multiple dose study of ARO-ANG3, a subcutaneously administered RNAi therapeutic targeting angiopoietin like protein 3, being developed as a potential treatment for patients with dyslipidemias and metabolic diseases
- Began dosing in a Phase 1 single and multiple dose study of ARO-APOC3, a subcutaneously administered RNAi therapeutic targeting apolipoprotein C-III, being developed as a potential treatment for patients with hypertriglyceridemia
- Received FDA clearance to begin an adaptive design Phase 2/3 trial, now 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 (AAT) deficiency
- Presented data on both the AAT and hepatitis B programs at the EASL International Liver Congress™ 2019. Key findings included the following:
  - Sustained RNAi reduction of the mutant Z-AAT protein in PiZ mice, which harbor the human Z-AAT gene and recapitulate many features of human AAT deficiency liver disease, treated for 33 weeks substantially reversed the disease phenotype
  - JNJ-3989, formerly ARO-HBV, rapidly reduced hepatitis B surface antigen (HBsAg) in patients that had 24 weeks or more of HBsAg assay results (n=40) to thresholds possibly associated with improved chances of HBsAg seroclearance in many patients, after only 3 doses
    - 100% of patients (40 of 40) achieved ≥1.0 Log10 IU/mL HBsAg reduction
    - 88% of patients (35 of 40) achieved HBsAg <100 IU/mL
  - JNJ-3989 reduced all measurable viral products, including HBsAg in hepatitis B e-antigen (HBeAg) positive or HBeAg negative patients
  - JNJ-3989 administered subcutaneously was well tolerated at doses up to 400 mg in all chronic hepatitis B patients
- Expanded the AROHBV1001 Phase 1/2 study to include a new triple combination cohort that includes JNJ-3989 and additional undisclosed agents selected by Arrowhead's partner, Janssen Pharmaceuticals, Inc.
  - In connection with the start of dosing of this cohort, Arrowhead earned a $25 million milestone payment from Janssen

Selected Fiscal 2019 Second Quarter Financial Results

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

	Three Months Ended March 31,		Six Months Ended March 31,	
OPERATING SUMMARY	**2019**	**2018**	**2019**	**2018**
REVENUE	**$ 48,148,275**	**$ 650,125**	**$ 82,806,171**	**$ 4,159,946**
OPERATING EXPENSES				
Research and development	20,798,628	12,002,354	38,370,671	24,921,972

General and administrative expenses	5,338,955	3,681,830	11,478,664	8,085,381
TOTAL OPERATING EXPENSES	26,137,583	15,684,184	49,849,335	33,007,353
OPERATING INCOME (LOSS)	**22,010,692**	**(15,034,059)**	**32,956,836**	**(28,847,407)**
OTHER INCOME/(EXPENSE), PROVISION FOR INCOME TAXES	**1,886,290**	**149,748**	**2,977,399**	**764,218**
NET INCOME (LOSS)	**$ 23,896,982**	**$ (14,884,311)**	**$ 35,934,235**	**$ (28,083,189)**
NET INCOME (LOSS) PER SHARE (DILUTED)	**$ 0.24**	**$ (0.18)**	**$ 0.37**	**$ (0.35)**
WEIGHTED AVERAGE SHARES OUTSTANDING (DILUTED)	**98,082,644**	**84,083,937**	**97,214,546**	**79,406,838**

FINANCIAL POSITION SUMMARY	**March 31,**	**September 30,**
	2019	**2018**
CASH AND CASH EQUIVALENTS	**$ 161,639,208**	**$ 30,133,213**
SHORT-TERM INVESTMENTS	**56,560,674**	**46,400,176**
LONG-TERM INVESTMENTS	**67,536,054**	**-**
TOTAL CASH RESOURCES (CASH AND INVESTMENTS)	**285,735,936**	**76,533,389**
OTHER ASSETS	**48,204,497**	**35,076,562**
TOTAL ASSETS	**333,940,433**	**111,609,951**
TOTAL CURRENT DEFERRED REVENUE	**90,889,801**	**600**
TOTAL LONG TERM DEFERRED REVENUE	**29,732,938**	**-**
OTHER LIABILITIES	**12,677,458**	**16,368,350**
TOTAL LIABILITIES	**133,300,197**	**16,368,950**
TOTAL STOCKHOLDERS' EQUITY	**200,640,236**	**95,241,001**
TOTAL LIABILITIES AND STOCKHOLDERS' EQUITY	**$ 333,940,433**	**$ 111,609,951**
SHARES OUTSTANDING	**94,665,718**	**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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