Arrowhead Pharmaceuticals Reports Fiscal 2018 Second Quarter Results

May 8, 2018

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

PASADENA, Calif.--(BUSINESS WIRE)--May 8, 2018-- Arrowhead Pharmaceuticals Inc. (NASDAQ: ARWR) today announced financial results for its fiscal 2018 second quarter ended March 31, 2018. 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 2895628.

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 2895628.

Selected Fiscal 2018 Second Quarter and Recent Events

- Strengthened the balance sheet with an equity financing yielding gross proceeds of $60.4 million
- Received orphan drug designation from the United States Food and Drug Administration (FDA) for ARO-AAT, Arrowhead's second-generation investigational medicine for the treatment of a rare genetic liver disease associated with alpha-1 antitrypsin deficiency
- Initiated dosing in AROAAT1001 (NCT03362242), a Phase 1 single- and multiple-ascending dose study to evaluate the safety, tolerability, pharmacokinetics, and effect of ARO-AAT on serum alpha-1 antitrypsin levels in healthy adult volunteers
- Initiated dosing in AROHBV1001 (NCT03365947), a Phase 1/2 study to evaluate the safety, tolerability, and pharmacokinetic effects of single-ascending doses (SAD) of ARO-HBV in healthy adult volunteers, and to evaluate the safety, tolerability, and pharmacodynamic effects of multiple-ascending doses (MAD) of ARO-HBV in patients with chronic HBV
- Presented clinical data on ARC-520, the company's prior generation investigational medicine for the treatment of chronic hepatitis B infection, at The International Liver Congress™ 2018 (ILC), the annual meeting of the European Association for the Study of the Liver (EASL), including the following key results:
    - Multiple doses of ARC-520 resulted in s-antigen reductions in all patients by as much as 5.3 Log10
    - Where measurable, multi-log reductions were also seen in e-antigen, core-related antigen, DNA and HBV RNA
    - One e-antigen negative patient, while remaining on entecavir, serocleared for all measurable viral markers including s-antigen, core-related antigen, HBV RNA, and HBV DNA. We believe this will represent a functional cure
    - 2 out of 3 e-antigen positive and 2 out of 5 e-antigen negative patients, or half of the patients in the study, achieved productive and sustained host responses. These were characterized by mild ALT elevations coinciding with continued reductions in various viral markers which persisted after ARC-520 therapy was removed
    - Two patients that experienced sustained host responses but had not yet serocleared, appear poised to potentially seroclear if the trends in the decrease of viral markers continues
- Presented preclinical data on both ARO-AAT and ARO-HBV at EASL
- Made continued progress on the emerging pipeline of RNAi therapeutics developed using the Targeted RNAi Molecule (TRiM™) platform including:
    - The cardiometabolic pipeline, which includes ARO-APOC3, targeting apolipoprotein C-III (ApoC3), and ARO-ANG3, targeting angiopoietin-like protein 3 (ANGPTL3)
    - The pulmonary pipeline, which includes ARO-ENaC, formerly called ARO-Lung1, which is an inhaled RNAi therapeutic targeting the epithelial sodium channel alpha subunit (αENaC) for the treatment of cystic fibrosis
    - ARO-HIF2 for the treatment of clear cell renal cell carcinoma

Selected Fiscal 2018 Second Quarter Financial Results

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

	Three Months Ended	Six Months Ended
	March 31,	March 31,

OPERATING SUMMARY	2018	2017	2018	2017
REVENUE	**$ 650,125**	**$ 8,985,930**	**$ 4,159,946**	**$ 13,351,426**
OPERATING EXPENSES				
Research and development	12,002,354	11,438,216	24,921,972	26,226,466
General and administrative expenses	3,681,830	3,677,356	8,085,381	8,156,491
TOTAL OPERATING EXPENSES	15,684,184	15,115,572	33,007,353	34,382,957
OPERATING LOSS	**(15,034,059)**	**(6,129,642)**	**(28,847,407)**	**(21,031,531)**
OTHER INCOME/(EXPENSE), PROVISION FOR INCOME TAXES	**149,748**	**87,085**	**764,218**	**2,902,864**
NET LOSS	**$ (14,884,311)**	**$ (6,042,557)**	**$ (28,083,189)**	**$ (18,128,667)**
NET LOSS PER SHARE (BASIC AND DILUTED):	**$ (0.18)**	**$ (0.08)**	**$ (0.35)**	**$ (0.25)**
WEIGHTED AVERAGE SHARES OUTSTANDING	**84,083,937**	**74,629,855**	**79,406,838**	**73,019,726**

FINANCIAL POSITION SUMMARY	March 31, 2018	September 30, 2017
CASH AND CASH EQUIVALENTS	**$ 69,805,117**	**$ 24,838,567**
SHORT-TERM INVESTMENTS	**21,736,820**	**40,769,539**
TOTAL CASH RESOURCES (CASH AND INVESTMENTS)	**91,541,937**	**65,608,106**
OTHER ASSETS	**35,456,978**	**38,414,174**
TOTAL ASSETS	**126,998,915**	**104,022,280**
TOTAL LIABILITIES	**13,283,833**	**23,155,118**
TOTAL STOCKHOLDERS' EQUITY	**113,715,082**	**80,867,162**
TOTAL LIABILITIES AND STOCKHOLDERS' EQUITY	**$ 126,998,915**	**$ 104,022,280**
SHARES OUTSTANDING	**87,570,398**	**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. 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 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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