Arrowhead Pharmaceuticals Reports Fiscal 2021 Third Quarter Results

August 5, 2021

Conference Call and Webcast Today, August 5, 2021 at 4:30 p.m. ET

PASADENA, Calif.--(BUSINESS WIRE)--Aug. 5, 2021-- Arrowhead Pharmaceuticals, Inc. (NASDAQ: ARWR) today announced financial results for its fiscal third quarter ended June 30, 2021. The company is hosting a conference call today, August 5, 2021, at 4:30 p.m. ET to discuss the 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 7398304.

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 7398304.

Selected Recent Events

- Received Breakthrough Therapy designation from the U.S. Food and Drug Administration for ARO-AAT, also known as TAK-999, the company's second-generation investigational RNA interference (RNAi) therapeutic being co-developed with Takeda Pharmaceutical Company Limited as a treatment for the rare genetic liver disease associated with alpha-1 antitrypsin deficiency.
- Presented additional positive interim 48-week liver biopsy results from the ongoing AROAAT2002 study, an open-label Phase 2 clinical study of ARO-AAT, at The International Liver Congress - The Annual Meeting of the European Association for the Study of the Liver (EASL). The results demonstrate that investigational ARO-AAT treatment led to improvements in multiple measures of liver health, including fibrosis, with substantial and sustained reductions in the level of mutant AAT protein. In addition, ARO-AAT treatment was generally well tolerated after up to 1 year of treatment.
- Presented positive interim results from AROHSD1001, an ongoing Phase 1/2 clinical study of ARO-HSD, the company's investigational RNAi therapeutic being developed as a treatment for patients with alcohol-related and nonalcohol related liver diseases, such as nonalcoholic steatohepatitis (NASH), at EASL. The data demonstrate that ARO-HSD is the first investigational therapeutic to achieve robust reductions in messenger RNA and protein levels of hepatic HSD17B13, leading to reductions in alanine aminotransferase (ALT), a liver enzyme typically elevated in liver diseases including NASH.
- Announced positive interim results from the first two cohorts of AROHIF21001, a Phase 1b dose-finding clinical study of ARO-HIF2, the company's investigational RNAi therapeutic being developed as a treatment for patients with clear cell renal cell carcinoma. The data show clear signs of meaningful target engagement and some potentially early signs of efficacy in at least one patient.
- Initiated and began dosing patients in AROANG3-2001, a Phase 2b clinical study of ARO-ANG3, the company's investigational RNAi therapeutic being developed as a treatment for patients with mixed dyslipidemia.
- Initiated and began dosing patients in AROAPOC3-2001, a Phase 2b clinical study of ARO-APOC3, the company's investigational RNA interference (RNAi) therapeutic being developed as a treatment for patients with severe hypertriglyceridemia (SHTG). Arrowhead also intends to initiate a Phase 2b study and a Phase 3 study of ARO-APOC3 in two additional patient populations in 2021.
- Announced a global collaboration and license agreement with Horizon Therapeutics for ARO-XDH, a previously undisclosed discovery-stage RNAi therapeutic being developed by Arrowhead as a potential treatment for people with uncontrolled gout. Arrowhead received $40 million as an upfront payment from Horizon and is eligible to receive up to $660 million in potential development, regulatory and commercial milestones, and is further eligible to receive royalties in the low- to mid-teens range on net product sales.
- Earned a $10 million option exercise fee from Janssen Pharmaceuticals, Inc., part of the Janssen Pharmaceutical Companies of Johnson & Johnson, for ARO-JNJ1.
- Presented promising preclinical data on ARO-DUX4, Arrowhead's first muscle-targeted program being developed as a treatment for patients with facioscapulohumeral muscular dystrophy (FSHD) at the 28th Annual FSHD Society International Research Congress. The data show that the TRiM^{TM} muscle delivery platform achieved functional delivery to various types of skeletal muscle and achieved deep, durable, and dose-dependent knockdown of target genes. In addition, ARO-DUX4 improved multiple measures of FSHD-like muscle phenotype in relevant preclinical animal models.
- Nominated ARO-C3, which is designed to reduce production of complement component 3 (C3) as a potential treatment for various complement mediated diseases, as a clinical candidate and initiated IND-enabling toxicology studies.

Selected Fiscal 2021 Third Quarter Financial Results

ARROWHEAD PHARMACEUTICALS, INC.
CONSOLIDATED CONDENSED FINANCIAL INFORMATION (unaudited)
(in thousands, except per share amounts)

	Three months ended June 30,		Nine months ended June 30,	
OPERATING SUMMARY	**2021**	**2020**	**2021**	**2020**
REVENUE	**$ 45,891**	**$ 27,376**	**$ 100,004**	**$ 80,359**
OPERATING EXPENSES				
Research and development	59,325	32,573	140,576	85,390
General and administrative expenses	18,434	10,749	43,581	38,009
TOTAL OPERATING EXPENSES	77,759	43,322	184,157	123,399
OPERATING INCOME (LOSS)	**(31,868)**	**(15,946)**	**(84,153)**	**(43,040)**
OTHER INCOME/(EXPENSE)	**1,944**	**2,335**	**6,679**	**6,920**
NET INCOME (LOSS)	**$ (29,924)**	**$ (13,611)**	**$ (77,474)**	**$ (36,120)**
NET INCOME (LOSS) PER SHARE (DILUTED)	**$ (0.29)**	**$ (0.13)**	**$ (0.75)**	**$ (0.36)**
WEIGHTED AVERAGE SHARES OUTSTANDING (DILUTED)	**104,099**	**101,843**	**103,569**	**100,184**

FINANCIAL POSITION SUMMARY	**June 30, 2021**	**September 30, 2020**
CASH AND CASH EQUIVALENTS	**$ 325,981**	**$ 143,583**
SHORT-TERM INVESTMENTS AND MARKETABLE SECURITIES	**190,331**	**171,910**
LONG-TERM INVESTMENTS	**128,376**	**137,487**
TOTAL CASH RESOURCES (CASH AND INVESTMENTS)	**644,688**	**452,980**
OTHER ASSETS	**90,239**	**69,524**
TOTAL ASSETS	**734,927**	**522,504**
TOTAL CURRENT DEFERRED REVENUE	**150,934**	**19,291**
OTHER LIABILITIES	**147,103**	**41,434**
TOTAL LIABILITIES	**298,037**	**60,725**
TOTAL STOCKHOLDERS' EQUITY	**436,890**	**461,779**
TOTAL LIABILITIES AND STOCKHOLDERS' EQUITY	**$ 734,927**	**$ 522,504**
SHARES OUTSTANDING	**104,209**	**102,376**

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. Any statements contained in this release except for historical information may be deemed to be forward-looking statements. Without limiting the generality of the foregoing, words such as "may," "will," "expect," "believe," "anticipate," "hope," "intend," "plan," "project," "could," "estimate," or "continue" are intended to identify such forward-looking statements. In addition, any statements that refer to projections of our future financial performance, trends in our business, expectations for our product pipeline or product candidates, including anticipated regulatory submissions and clinical program results, prospects or benefits of our collaborations with other companies, or other characterizations of future events or circumstances are forward-looking statements. 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 numerous factors and uncertainties, including the impact of the ongoing COVID-19 pandemic on our business, the safety and efficacy of our product candidates, decisions of regulatory authorities and the timing thereof, 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, the enforcement of our intellectual property rights, and the other risks and uncertainties described in our most recent Annual Report on Form 10-K, subsequent Quarterly Reports on Form 10-Q and other documents filed with the Securities and Exchange Commission from time to time. We assume no obligation to update or revise forward-looking statements to reflect new events or circumstances.

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