



Arrowhead Pharmaceuticals Reports Fiscal 2021 Second Quarter Results

May 4, 2021

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

PASADENA, Calif.--(BUSINESS WIRE)--May 4, 2021-- Arrowhead Pharmaceuticals, Inc. (NASDAQ: ARWR) today announced financial results for its fiscal second quarter ended March 31, 2021. The company is hosting a conference call today, May 4, 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 3080187.

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 3080187.

Selected Recent Events

- Announced positive interim 48-week liver biopsy results from the AROAAT2002 study, an open-label Phase 2 clinical study of ARO-AAT, 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 (AATD). The interim readout demonstrated that ARO-AAT treatment led to:
 - Consistent and substantial reduction in intra-hepatic mutant AAT protein (Z-AAT), both Z-AAT monomer and Z-AAT polymer
 - Consistent decrease in histological globule burden
 - Improvements in fibrosis
 - Improvements in other relevant biomarkers of liver health
- Arrowhead intends to present additional interim AROAAT2002 data at an upcoming medical congress, pending abstract acceptance.
- Announced ARO-DUX4, Arrowhead's first muscle targeted investigational RNAi therapeutic candidate to utilize its proprietary Targeted RNAi Molecule (TRIM™) platform. ARO-DUX4 is designed to target the gene that encodes human double homeobox 4 (DUX4) protein as a potential treatment for patients with facioscapulohumeral muscular dystrophy (FSHD).
- Submitted an Investigational New Drug Application (IND) to the U.S. Food and Drug Administration (FDA) for a Phase 2b dose-finding clinical study of ARO-ANG3, the company's investigational RNAi therapeutic being developed as a treatment for patients with mixed dyslipidemia.
- Submitted an IND to the FDA for a Phase 2b dose-finding clinical study of ARO-APOC3, the company's investigational RNAi therapeutic being developed as a treatment for patients with hypertriglyceridemia.

Selected Fiscal 2021 Second Quarter Financial Results

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

	Three months ended March 31,		Six months ended March 31,	
	2021	2020	2021	2020
OPERATING SUMMARY				
REVENUE	\$ 32,811	\$ 23,529	\$ 54,113	\$ 52,983
OPERATING EXPENSES				
Research and development	44,697	29,443	81,251	52,817
General and administrative expenses	16,346	16,326	25,147	27,260
TOTAL OPERATING EXPENSES	61,043	45,769	106,398	80,077
OPERATING INCOME (LOSS)	(28,232)	(22,240)	(52,285)	(27,094)
OTHER INCOME/(EXPENSE)	1,414	2,404	4,735	4,585
NET INCOME (LOSS)	\$ (26,818)	\$ (19,836)	\$ (47,550)	\$ (22,509)
NET INCOME (LOSS) PER SHARE (DILUTED)	\$ (0.26)	\$ (0.20)	\$ (0.46)	\$ (0.23)

WEIGHTED AVERAGE SHARES OUTSTANDING (DILUTED)	<u>103,867</u>	<u>101,653</u>	<u>103,303</u>	<u>99,359</u>
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FINANCIAL POSITION SUMMARY

	March 31, 2021	September 30, 2020
CASH AND CASH EQUIVALENTS	\$ 372,377	\$ 143,583
SHORT-TERM INVESTMENTS AND MARKETABLE SECURITIES	204,941	171,910
LONG-TERM INVESTMENTS	97,490	137,487
TOTAL CASH RESOURCES (CASH AND INVESTMENTS)	<u>674,808</u>	<u>452,980</u>
OTHER ASSETS	81,987	69,524
TOTAL ASSETS	<u>756,795</u>	<u>522,504</u>
TOTAL CURRENT DEFERRED REVENUE	144,879	19,291
OTHER LIABILITIES	166,367	41,434
TOTAL LIABILITIES	<u>311,246</u>	<u>60,725</u>
TOTAL STOCKHOLDERS' EQUITY	<u>445,549</u>	<u>461,779</u>
TOTAL LIABILITIES AND STOCKHOLDERS' EQUITY	<u>\$ 756,795</u>	<u>\$ 522,504</u>
SHARES OUTSTANDING	104,020	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](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. 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," "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 businesses, 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 continuing impact of the COVID-19 pandemic, 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, 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.

Source: Arrowhead Pharmaceuticals, Inc.

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