



Arrowhead Pharmaceuticals Reports Fiscal 2023 Third Quarter Results

August 7, 2023

Conference Call and Webcast Today, August 7, 2023 at 4:30 p.m. ET

PASADENA, Calif.--(BUSINESS WIRE)--Aug. 7, 2023-- Arrowhead Pharmaceuticals, Inc. (NASDAQ: ARWR) today announced financial results for its fiscal third quarter ended June 30, 2023. The company is hosting a conference call today, Aug. 7, 2023, at 4:30 p.m. ET to discuss the results.

Webcast and Conference Call and Details

Investors may access a live audio webcast on the Company's website at <http://ir.arrowheadpharma.com/events.cfm>. A replay of the webcast will be available approximately two hours after the conclusion of the call.

For analysts that wish to participate in the conference call, please register at <https://register.vevent.com/register/Blcd0f2b791a4f4cdd97a1c78e09086106>. Once registered, you will receive the dial-in number and a personalized PIN code that will be required to access the call.

Selected Recent Events

- Hosted a Research & Development Day to discuss progress towards the company's "20 in 25" goal to grow its pipeline of RNAi therapeutics that leverage the proprietary Targeted RNAi Molecule (TRiM™) platform to a total of 20 clinical stage or marketed products in the year 2025. Key updates included:
 - New Phase 1 clinical data on ARO-RAGE showing a continued dose response in healthy volunteers with a single inhaled dose of 184 mg achieving mean knockdown of 90% and a maximum of 95% in bronchoalveolar lavage fluid (BALF)
 - Serum sRAGE reductions in asthma patients consistent with effects seen in healthy volunteers at the 44 mg dose, the only dose level with results currently available in asthma patient cohorts
 - TRiM™ platform for intrathecal administration for central nervous system (CNS) delivery demonstrates 90-95% dose-dependent mRNA knockdown in disease-relevant spinal cord and cortex brain regions in non-human primates
 - TRiM™ platform for adipose tissue delivery achieved up to 98% knockdown and maintained greater than 85% knockdown over 31 weeks
 - TRiM™ dimer platform for delivery to hepatocytes achieved equivalent or better knockdown of two separate target genes with longer duration than monomer mixture
- Completed enrollment of the global PALISADE Phase 3 clinical trial evaluating ARO-APOC3 for the treatment of familial chylomicronemia syndrome. The company anticipates that the primary portion of the study will be complete in the second quarter of 2024 with a data readout shortly thereafter
- Presented interim data from the ongoing Phase 2 GATEWAY clinical study of ARO-ANG3 in patients with homozygous familial hypercholesterolemia at the 91st European Atherosclerosis Society Congress showing that ARO-ANG3 achieved 44-48% mean reductions in LDL-C on top of continued standard of care
- Presented updated results from the Phase 2 SEQUOIA clinical study of investigational fazirsiran (TAK-999/ARO-AAT) for the treatment of liver disease associated with alpha-1 antitrypsin deficiency at the European Association for the Study of the Liver (EASL) Congress 2023
- Filed an application to initiate a Phase 1 clinical trial of ARO-SOD1, an RNAi-based investigational medicine designed to reduce expression of superoxide dismutase 1 (SOD1) in the CNS as a potential treatment for patients with amyotrophic lateral sclerosis (ALS) caused by SOD1 mutations. ARO-SOD1 is the first therapeutic candidate designed for delivery to the CNS to enter clinical studies that leverages the TRiM™ platform
- Filed an application to initiate a Phase 1/2 clinical trial of ARO-DUX4, the company's investigational RNAi therapeutic being developed as a potential treatment for patients with facioscapulohumeral muscular dystrophy (FSHD). ARO-DUX4 is the first clinical candidate utilizing the TRiM™ platform to target disease associated genes in skeletal muscle
- Earned a \$30 million milestone payment from GSK (LSE/NYSE: GSK) following the start of GSK's Phase 2b trial of GSK4532990, formerly called ARO-HSD, an investigational RNAi therapeutic for the treatment of patients with non-alcoholic steatohepatitis (NASH)
- Earned a \$40 million milestone payment from Takeda (TSE:4502/NYSE:TAK) after the first patient was dosed in the Phase 3 REDWOOD clinical study of fazirsiran

Selected Fiscal 2023 Third Quarter Financial Results

Three Months Ended June 30,

OPERATING SUMMARY

	2023	2022
	(unaudited)	
Revenue	\$ 15,825	\$ 32,412
Operating Expenses:		
Research and development	94,757	72,180
General and administrative expenses	23,771	33,141
Total Operating Expenses	118,528	105,321
Operating loss	(102,703)	(72,909)
Interest income	4,172	1,240
Interest expense	(5,158)	-
Other, net	306	(377)
Loss before income tax expense and noncontrolling interest	(103,383)	(72,046)
Income tax expense	742	-
Net loss before noncontrolling interest	(104,125)	(72,046)
Net loss attributable to noncontrolling interest, net of tax	(1,179)	-
Net loss attributable to Arrowhead Pharmaceuticals, Inc.	\$ (102,946)	\$ (72,046)
Net loss per share attributable to Arrowhead Pharmaceuticals, Inc. - Diluted	\$ (0.96)	\$ (0.68)
Weighted-average shares used in calculating - Diluted	107,004	105,753

FINANCIAL POSITION SUMMARY

	June 30, 2023	September 30, 2022
	(unaudited)	
Cash, cash equivalents and restricted cash	\$ 105,334	\$ 108,005
Short-term investments	346,369	268,391
Long-term investments	42,758	105,872
Total cash resources (cash and investments)	494,461	482,268
Other assets	301,395	209,671
Total Assets	\$ 795,856	\$ 691,939
Current deferred revenue	\$ 16,905	\$ 74,099
Long-term deferred revenue	-	55,950
Other liabilities	396,966	143,551
Total Liabilities	\$ 413,871	\$ 273,600
Total Arrowhead Pharmaceuticals, Inc. Stockholders' Equity	\$ 364,830	\$ 398,520
Noncontrolling Interest	17,155	19,819
Total Noncontrolling Interest and Stockholders' Equity	381,985	418,339
Total Liabilities, Noncontrolling Interest and Stockholders' Equity	\$ 795,856	\$ 691,939
Shares Outstanding	107,102	105,960

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," "hope," "intend," "plan," "project," "could," "estimate," "continue," "target," "forecast" or "continue" or the negative of these words or other variations thereof or comparable terminology 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 forward-looking statements include, but are not limited to, statements about the initiation, timing, progress and results of our preclinical studies and clinical trials, and our research and development programs; our expectations regarding the potential benefits of the partnership, licensing and/or collaboration arrangements and other strategic arrangements and transactions we have entered into or may enter into in the future; our beliefs and expectations regarding milestone, royalty or other payments that could be due to or from third parties under existing agreements; and

our estimates regarding future revenues, research and development expenses, capital requirements and payments to third parties. 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.

Source: Arrowhead Pharmaceuticals, Inc.

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