Arrowhead Pharmaceuticals Reports Fiscal 2023 Second Quarter Results

May 2, 2023

- Conference Call and Webcast Today, May 2, 2023 at 4:30 p.m. ET

PASADENA, Calif.--(BUSINESS WIRE)--May 2, 2023-- Arrowhead Pharmaceuticals, Inc. (NASDAQ: ARWR) today announced financial results for its fiscal second quarter ended March 31, 2023. The company is hosting a conference call today, May 2, 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/BIf9cd65655e5444119df91cd4891157f8. 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

- Reported interim results from an ongoing Phase 1/2 clinical study of ARO-RAGE, an investigational RNAi therapeutic for treatment for inflammatory pulmonary diseases, such as asthma. These data represent the first clinical demonstration of the potential utility of Arrowhead's proprietary Targeted RNAi Molecule (TRiM ™) platform optimized for delivery to the lungs. Key results from Part 1 of the study in normal healthy volunteers included the following:
  - Mean maximum reduction in soluble RAGE (sRAGE) as measured in serum after two doses of 92 mg on Day 1 and Day 29 was 80% with a maximum reduction of 90%
    - Duration of pharmacologic effect persisted for at least 6 weeks
  - Mean reduction in sRAGE as measured in bronchoalveolar lavage fluid (BALF) at Day 31 after a single dose of 92 mg was 75% with a maximum reduction of 92%
  - Mean maximum reduction of 56% with a maximum reduction of 68% in serum sRAGE was also observed after a single dose of 92 mg
  - Safety and tolerability
    - Overall, no patterns of adverse changes in any clinical safety parameters
    - No reported serious or severe adverse events
    - No dropouts related to drug or related to adverse events
  - These results include 4 of 5 escalating dose levels. Data are not yet available for single or multiple dose cohorts at 184 mg, the highest dose being tested
- Announced a planned R&D Day on June 1, 2023, which will include presentations on cardiometabolic, pulmonary, and newly announced central nervous system (CNS) pipeline programs
  - The company expanded its TRiM™ platform to include an optimized intrathecal administration for CNS delivery with good distribution throughout the brain and in all relevant brain cell types. The first development candidate to utilize this new delivery platform, ARO-SOD1, is on track for a clinical trial application filing in the third quarter of 2023 to begin clinical studies
  - In preclinical studies, ARO-SOD1 achieved 95% spinal cord tissue mRNA knockdown after a single intrathecal dose in human SOD1 transgenic rats and maintained greater than 80% spinal cord tissue mRNA knockdown three months after a single intrathecal dose in non-human primates
- 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 (TAK-999/ARO-AAT) for the treatment of alpha-1 antitrypsin deficiency associated liver disease (AATD-LD)
- Received Fast Track designation from the U.S. Food and Drug Administration (FDA) for ARO-APOC3 for reducing triglycerides in adult patients with familial chylomicronemia syndrome
- 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)
- Initiated dosing in a Phase 1/2a single ascending dose and multiple ascending dose clinical study of ARO-MMP7, Arrowhead's third pulmonary targeted investigational RNAi therapeutic being developed as a potential treatment for idiopathic pulmonary fibrosis
- Reported interim results from an ongoing Phase 1/2 clinical study of ARO-C3, an investigational RNAi therapeutic for treatment of various complement mediated diseases, demonstrating mean reductions of 88% in C3 and 91% in AH50, a marker of alternative complement pathway hemolytic activity
- Regained rights to ARO-PNPLA3, formerly called JNJ-75220795, which was part of a 2018 research collaboration and option agreement between Arrowhead and Janssen Pharmaceuticals, Inc. ARO-PNPLA3 is an investigational RNAi therapeutic developed using Arrowhead's proprietary TRiM ™ platform and designed to reduce liver expression of patatin-like phospholipase domain containing 3 (PNPLA3) as a potential treatment for patients with NASH

- Announced topline results with Takeda from the Phase 2 SEQUOIA clinical study of investigational fazirsiran for the treatment of AATD-LD and provided an outline of a Phase 3 study that was co-developed by Takeda and Arrowhead and will be conducted by Takeda. Key results from SEQUOIA included the following:
  - Fibrosis regression observed in 50% of patients receiving fazirsiran
  - Median reductions of 94% of Z-AAT accumulation in the liver and mean reduction of 68% in histologic globule burden
  - Treatment emergent adverse events were generally well balanced between fazirsiran and placebo groups
  - Results consistent with AROAAT-2002 open-label study previously published in *The New England Journal of Medicine*

Selected Fiscal 2023 Second Quarter Financial Results

OPERATING SUMMARY	**Three Months Ended March 31,**	
	2023	**2022**
	(unaudited)	
Revenue	**$ 146,267**	**$ 151,805**
Operating Expenses:		
Research and development	74,881	75,985
General and administrative expenses	23,221	34,267
Total Operating Expenses	98,102	110,252
Operating income	48,165	41,553
Interest income	4,560	1,054
Interest expense	(5,067)	-
Other, net	8	1,759
Income before income tax expense and noncontrolling interest	47,676	44,366
Income tax expense	-	-
Net income before noncontrolling interest	47,676	44,366
Net loss attributable to noncontrolling interest, net of tax	(999)	-
Net income attributable to Arrowhead Pharmaceuticals, Inc.	**$ 48,675**	**$ 44,366**
Net income per share attributable to Arrowhead Pharmaceuticals, Inc. - Diluted	**$ 0.45**	**$ 0.41**
Weighted-average shares used in calculating - Diluted	108,143	107,929

FINANCIAL POSITION SUMMARY	**March 31, 2023**	**September 30, 2022**
	(unaudited)	
Cash, cash equivalents and restricted cash	$ 134,959	$ 108,005
Short-term investments	346,046	268,391
Long-term investments	78,834	105,872
Total cash resources (cash and investments)	**559,839**	**482,268**
Other assets	331,469	209,671
Total Assets	**$ 891,308**	**$ 691,939**
Current deferred revenue	$ 29,839	$ 74,099
Long-term deferred revenue	1,075	55,950
Other liabilities	395,288	143,551
Total Liabilities	**$ 426,202**	**$ 273,600**
Total Arrowhead Pharmaceuticals, Inc. Stockholders' Equity	**$ 446,772**	**$ 398,520**
Noncontrolling Interest	18,334	19,819
Total Noncontrolling Interest and Stockholders' Equity	**465,106**	**418,339**
Total Liabilities, Noncontrolling Interest and Stockholders' Equity	**$ 891,308**	**$ 691,939**
Shares Outstanding	106,869	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. 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.

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