Arrowhead Pharmaceuticals Reports Fiscal 2025 First Quarter Results

February 10, 2025

- Conference Call and Webcast Today, February 10, 2025, at 4:30 p.m. ET

PASADENA, Calif.--(BUSINESS WIRE)--Feb. 10, 2025-- Arrowhead Pharmaceuticals, Inc. (NASDAQ: ARWR) today announced financial results for its fiscal 2025 first quarter ended December 31, 2024. The Company is hosting a conference call today, February 10, 2025, at 4:30 p.m. ET to discuss the results.

“During the recent period, Arrowhead signed and closed a potentially transformational licensing and collaboration agreement with Sarepta Therapeutics and submitted our first NDA for investigational plozasiran, which was subsequently accepted for filing by the U.S. FDA,” said Christopher Anzalone, Ph.D., President and CEO at Arrowhead. “The company is now well positioned for growth with plans for an independent commercial launch in 2025 and the potential for multiple partner launches over the coming few years. Phase 3 studies of plozasiran in severe hypertriglyceridemia are on pace to be fully enrolled in 2025 with potential study completion in 2026. We see emerging high-value potential in our obesity and CNS programs entering early clinical studies. Arrowhead is currently funded into 2028 with further cash runway potential with multiple wholly owned candidates providing opportunities for additional partnerships.”

Selected Recent Events

- Announced that the U.S. Food and Drug Administration (FDA) has accepted the New Drug Application (NDA) for investigational plozasiran for the treatment of familial chylomicronemia syndrome, a severe and rare genetic disease.
  - The clinical basis of the NDA submission is comprised of the positive findings from the Phase 3 PALISADE study with supportive confirmatory evidence from the Phase 2 clinical studies of the SUMMIT program.
  - PALISADE successfully met its primary endpoint and all multiplicity-controlled key secondary endpoints, including statistically significant reductions in triglycerides, apolipoprotein C-III, and the incidence of acute pancreatitis.
  - The FDA provided a Prescription Drug User Fee Act (PDUFA) action date of November 18, 2025, and indicated it is not currently planning to hold an advisory committee meeting.
- Signed and closed a licensing and collaboration agreement with Sarepta Therapeutics. Upon closing, Arrowhead receives $825 million, consisting of $500 million cash and $325 million as an equity investment priced at $27.2507, representing a 35% premium to the 30-day volume weighted average price. Arrowhead will also receive $250 million to be paid over five years. Arrowhead is also eligible to receive an additional $300 million in near-term payments, royalties on commercial sales, and approximately $10 billion in potential milestone payments.
- Announced new results from the Phase 3 PALISADE study and the open-label extension from the Phase 2 MUIR and SHASTA-2 studies of investigational plozasiran. These data were presented in two oral presentations at the American Heart Association Scientific Sessions 2024 (AHA24) and PALISADE data was simultaneously published in the AHA journal, *Circulation*. Key results include the following:
  - Plozasiran induced deep and sustained reductions in triglycerides and impacted a wide spectrum of lipoproteins that may be involved with atherosclerotic cardiovascular disease.
  - Similar responses were observed in patients with genetically confirmed and clinically diagnosed familial chylomicronemia syndrome.
  - Mean reductions from baseline in triglycerides of up to -73% in patients from MUIR and -86% in patients from SHASTA-2 with favorable reductions in remnant cholesterol and non-HDL-cholesterol were observed through 15 months follow up in the open-label extension.
  - Overall, plozasiran has been generally well-tolerated to date. In the PALISADE study, the most frequently reported treatment emergent adverse events for the 25 mg dose were abdominal pain, COVID-19, nasopharyngitis, and nausea. Across clinical studies and study populations, the most frequently reported treatment emergent adverse events for the 25 mg dose were COVID-19, upper respiratory tract infection, headache, Type 2 diabetes mellitus, and abdominal pain.
- Initiated a Phase 1/2a clinical trial of ARO-INHBE, the company’s investigational RNAi therapeutic being developed as a potential treatment for obesity.
  - ARO-INHBE targets a known pathway that signals the body to store fat in adipose tissue.
  - In preclinical studies, ARO-INHBE reduced body weight and fat mass with a novel mechanism of action that may better preserve lean muscle mass compared to currently approved obesity therapies.
- Filed for and recently received regulatory clearance in New Zealand to initiate a Phase 1/2a clinical trial of ARO-ALK7, the company’s investigational RNAi therapeutic being developed as a potential treatment for obesity.
- Presented interim results from a Phase 1/2a clinical study of ARO-CFB, the company’s investigational RNAi therapeutic targeting complement factor B being developed as a potential treatment for complement mediated diseases.
  - Demonstrated near complete inhibition in hemolytic activity and functional activity of the alternative complement pathway.

Selected Fiscal 2025 First Quarter Financial Results

ARROWHEAD PHARMACEUTICALS, INC.
CONSOLIDATED CONDENSED FINANCIAL INFORMATION

(in thousands, except per share amounts)

OPERATING SUMMARY

	Three Months December 31,	
	2024	2023
	(unaudited)	
Revenue	**$ 2,500**	**$ 3,551**
Operating Expenses:		
Research and development	137,002	116,491
General and administrative expenses	26,910	23,605
Total operating expenses	163,912	140,096
Operating loss	(161,412)	(136,545)
Total other expense	(13,703)	(2,144)
Loss before income tax expense and noncontrolling interest	(175,115)	(138,689)
Income tax expense (benefit)	103	(3,313)
Net loss including noncontrolling interest	(175,218)	(135,376)
Net loss attributable to noncontrolling interest, net of tax	(2,133)	(2,512)
Net loss attributable to Arrowhead Pharmaceuticals, Inc.	**$ (173,085)**	**$ (132,864)**
Net loss per share attributable to Arrowhead Pharmaceuticals, Inc. - Diluted	**$ (1.39)**	**$ (1.24)**
Weighted-average shares used in calculating - Diluted	124,848	107,415

FINANCIAL POSITION SUMMARY

	December 31, 2024	September 30, 2024
	(unaudited)	
Cash, cash equivalents and restricted cash	$ 53,889	$ 102,685
Investments	499,046	578,276
Total cash resources (cash and investments)	**552,935**	**680,961**
Other assets	460,759	458,841
Total Assets	**$ 1,013,694**	**$ 1,139,802**
Liability related to the sale of future royalties	$ 346,776	$ 341,361
Credit Facility	409,414	393,183
Other liabilities	201,429	214,195
Total Liabilities	**$ 957,619**	**$ 948,739**
Total Arrowhead Pharmaceuticals, Inc. Stockholders' Equity	**52,589**	**185,444**
Noncontrolling Interest	3,486	5,619
Total Noncontrolling Interest and Stockholders' Equity	**$ 56,075**	**$ 191,063**
Total Liabilities, Noncontrolling Interest and Stockholders' Equity	**$ 1,013,694**	**$ 1,139,802**
Shares Outstanding	125,572	124,376

Webcast and Conference Call and Details

Investors may access a live audio webcast on the Events and Presentations page under the Investors section of the Arrowhead website. 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/BI10095fd77113444788810d4774d5fae3. Once registered, you will receive the dial-in number and a personalized PIN code that will be required to access the call.

About Plozasiran

Plozasiran, previously called ARO-APOC3, is a first-in-class investigational RNA interference (RNAi) therapeutic designed to reduce production of apolipoprotein C-III (APOC3) which is a component of triglyceride rich lipoproteins (TRLs) and a key regulator of triglyceride metabolism. APOC3 increases triglyceride levels in the blood by inhibiting breakdown of TRLs by lipoprotein lipase and uptake of TRL remnants by hepatic receptors in the liver. The goal of treatment with plozasiran is to reduce the level of APOC3, thereby reducing triglycerides and restoring lipids to more normal levels.

In multiple clinical studies, investigational plozasiran has demonstrated reductions in triglycerides and multiple atherogenic lipoproteins in patients with familial chylomicronemia syndrome (FCS), severe hypertriglyceridemia (SHTG), and mixed hyperlipidemia. Plozasiran has been generally well tolerated to date with treatment emergent adverse events reported that generally reflect the comorbidities and underlying conditions of the study populations. Across clinical studies and study populations, the most frequently reported treatment emergent adverse events for the 25 mg dose were COVID-19, upper respiratory tract infection, headache, Type 2 diabetes mellitus, and abdominal pain.

Plozasiran is being investigated in the SUMMIT program of clinical studies, including the PALISADE Phase 3 study in patients with FCS, the SHASTA studies in patients with SHTG, and the MUIR and CAPITAN studies in patients with mixed hyperlipidemia.

Plozasiran in the treatment of patients with FCS has been granted Breakthrough Therapy Designation, Orphan Drug Designation, and Fast Track Designation by the U.S. Food and Drug Administration and Orphan Drug Designation by the European Medicines Agency. Investigational plozasiran has not been reviewed or approved to treat any disease.

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 X (formerly Twitter) at @ArrowheadPharma, LinkedIn, Facebook, and Instagram. 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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