Arrowhead Pharmaceuticals Announces Interim Topline Clinical Data for the First Dual-Functional RNAi Therapeutic for the Treatment of Mixed Hyperlipidemia

September 15, 2026

- ARO-DIMER-PA achieved deep and durable silencing of two genes with a single RNAi molecule

- Mean maximal single dose reductions in serum PCSK9 of 72% and APOC3 of 88%

- Clinical validation further demonstrates Arrowhead's position as the leading innovator in siRNA therapies -

PASADENA, Calif.--(BUSINESS WIRE)--Sep. 15, 2026-- Arrowhead Pharmaceuticals, Inc. (NASDAQ: ARWR) today announced interim topline data from a Phase 1/2a clinical trial of ARO-DIMER-PA, the company's first investigational RNA interference (RNAi) therapeutic dimer being developed as a potential treatment for atherosclerotic cardiovascular disease (ASCVD) due to mixed hyperlipidemia. ARO-DIMER-PA is designed to silence expression of both proprotein convertase subtilisin kexin 9 (PCSK9) and apolipoprotein C3 (APOC3) genes. Additional results will be submitted for presentation at an upcoming medical congress.

"With these interim topline results, Arrowhead's innovative and proprietary Targeted RNAi Molecule (TRiM™) platform has achieved clinical validation of its ability to target and silence two genes simultaneously in one molecule. This represents an important step forward and a first for the field of RNAi therapeutics. We look forward to presenting additional details on this groundbreaking advance at an upcoming medical congress," said Chris Anzalone, Ph.D., President and CEO at Arrowhead Pharmaceuticals. "This validation potentially expands the number of diseases and patients worldwide that may be helped by Arrowhead's RNAi therapies. ARO-DIMER-PA also fits well strategically with Arrowhead's growing focus on cardiometabolic diseases. It is complemented by our existing portfolio, which includes our first commercial product REDEMPLO® (plozasiran), now approved in the U.S., European Union, Canada, Australia, and China for the treatment of familial chylomicronemia syndrome (FCS), and investigational zodasiran, which is currently being studied in the global Phase 3 YOSEMITE clinical trial in patients with homozygous familial hypercholesterolemia (HoFH)."

James Hamilton, M.D., MBA, Chief Medical Officer and head of R&D at Arrowhead, added, "The relationship between low-density lipoprotein-cholesterol (LDL-C) and Apolipoprotein B (ApoB) reduction and cardiovascular risk is one of the most consistently replicated findings in modern medicine. Over the past three decades, numerous clinical studies have shown that lowering these markers translates into fewer heart attacks, strokes, and cardiovascular deaths. The early single dose data with ARO-DIMER-PA in patients with mixed hyperlipidemia give us a high degree of confidence that the robust reductions in LDL-C and ApoB that we have seen, along with robust reductions in additional atherogenic lipoproteins, have the potential to translate into a reliable clinical benefit in later stage studies."

Steven Nissen, M.D., Chief Academic Officer for the Heart and Vascular Institute at the Cleveland Clinic, the Lewis and Patricia Dickey Chair in Cardiovascular Medicine and Professor of Medicine at the Lerner College of Medicine, added, "Mixed hyperlipidemia is not adequately addressed by treating LDL-C alone. Even with intensive statin therapy and PCSK9 inhibitors, substantial ASCVD risk remains, and triglyceride-rich remnant lipoproteins may be an important part of that residual risk. This medication represents a new approach to targeting multiple lipid abnormalities with a single therapy."

Select ARO-DIMER-PA Phase 1/2a Interim Single Dose Results

- In participants with mixed hyperlipidemia, ARO-DIMER-PA single doses achieved dose-dependent mean maximal reductions in serum PCSK9 of 72% and APOC3 of 88%
- Silencing of both the PCSK9 and APOC3 genes led to favorable changes in lipids and lipoproteins including the following mean maximal reductions:
    - LDL-C reduction of 54%
    - Triglyceride (TG) reduction of 73%
    - Non-high density lipoprotein (non-HDL) cholesterol reduction of 61%
    - Apolipoprotein B (ApoB) reduction of 50%

Interim Safety and Tolerability

Single dose escalation has been completed through 400 mg. The most commonly reported treatment emergent adverse events (TEAE) were injection site events and headaches. No drug related SAEs were reported. The study is ongoing to assess safety and tolerability of multiple doses.

About Mixed Hyperlipidemia

Mixed hyperlipidemia is a highly prevalent disorder characterized by elevated levels of both LDL-C and TGs. It is a major risk factor for ASCVD, which is the leading cause of mortality worldwide and associated with substantial morbidity and healthcare costs. Despite the efficacy of LDL-C-lowering therapies in reducing ASCVD risk, there remains substantial residual risk in patients with mixed hyperlipidemia.

About ARO-DIMER-PA

ARO-DIMER-PA is a dual-functional RNAi molecule designed to silence expression of both PCSK9 and APOC3 genes in hepatocytes. Prior clinical experience with other investigational and approved agents suggests that PCSK9 and APOC3 inhibition may lead to robust reductions in LDL-C, TGs, triglyceride rich lipoprotein remnants, and total atherogenic lipoproteins.

ARO-DIMER-PA is the first clinical candidate designed to selectively silence the expression of two genes with a single RNAi molecule.

About the ARODIMER-PA-1001 Phase 1/2a Study

ARO-DIMER-PA-1001 (NCT07223658) is a Phase 1/2a placebo-controlled dose-escalating study to evaluate the safety, tolerability, pharmacokinetics, pharmacodynamics, and effects on LDL-C and TGs of single-dose ARO-DIMER-PA (part 1) and multiple doses of ARO-DIMER-PA (part 2) in up to 78 adult subjects with mixed hyperlipidemia.

About Arrowhead Pharmaceuticals

Arrowhead Pharmaceuticals (NASDAQ: ARWR) is a commercial-stage pharmaceutical company developing medicines that treat intractable diseases by silencing the genes that cause them, harnessing the natural RNA interference (RNAi) mechanism. The company has built a broad portfolio of clinical and commercial RNAi therapeutics through its industry-leading targeted RNAi molecule (TRiM™) platform, which can precisely silence genes in a wide range of cell types, including liver, lung, muscle, adipose, and central nervous system tissue. At Arrowhead, we rapidly advance potential best- and first-in-class RNAi treatments for diseases with significant unmet medical need, because every day matters to the patients we serve.

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.

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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, products or product candidate or other characterizations of future events or circumstances are forward-looking statements. These forward-looking statements include, but are not limited to, statements about our beliefs and expectations regarding the long-term impacts on patient health and the health care system; our beliefs and expectations regarding the pricing, value, or expected timing for availability of our drugs and drug candidates; and our beliefs and expectations around the potential uses and value of the TRiM™ platform. These statements are based upon our current expectations and speak only as of the date hereof. Actual results or outcomes may differ materially and adversely from those expressed in any forward-looking statements as a result of numerous factors and uncertainties the safety and efficacy of our products and product candidates, pricing and reimbursement decisions related to our products, demand for our products, 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, 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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Source: Arrowhead Pharmaceuticals, Inc.