Arrowhead Pharmaceuticals Announces FDA Approval of REDEMPLO® (plozasiran) to Reduce Triglycerides in Adults with Familial Chylomicronemia Syndrome (FCS)

November 18, 2025

- REDEMPLO is the first and only FDA-approved medicine to be studied in patients with genetically confirmed and clinically diagnosed FCS

- People living with FCS have extremely high triglyceride levels and a substantially higher risk of acute pancreatitis and related long-term complications, often resulting in a reduced quality of life

- The FDA approval is based on positive results from the Phase 3 PALISADE study where REDEMPLO significantly reduced triglycerides from baseline and lowered the numerical incidence of acute pancreatitis compared to placebo

- Arrowhead will host a conference call and webcast today at 1:30 p.m. ET

PASADENA, Calif.--(BUSINESS WIRE)--Nov. 18, 2025-- Arrowhead Pharmaceuticals, Inc. (NASDAQ: ARWR) today announced that the U.S. Food and Drug Administration (FDA) has approved REDEMPLO (plozasiran), a small interfering RNA (siRNA) medicine, as an adjunct to diet to reduce triglycerides in adults with familial chylomicronemia syndrome (FCS). FCS is a severe, rare disease, with an estimated 6,500 people in the U.S. living with genetic or clinical FCS, characterized by triglyceride levels that can be 10 to 100 times higher than normal leading to a substantially higher risk of developing acute, recurrent, and potentially fatal pancreatitis. REDEMPLO is the first and only FDA-approved siRNA medicine for people living with FCS and can be self-administered at home with a simple subcutaneous injection once every three months. REDEMPLO utilizes the proprietary and differentiated Targeted RNAi Molecule (TRiM™) platform and is Arrowhead's first FDA-approved medicine, marking a major milestone for the company as it transitions into commercial-stage.

This press release features multimedia. View the full release here: https://www.businesswire.com/news/home/20251112678197/en/

REDEMPLO packaging and pre-filled syringe

"The FDA approval of REDEMPLO is a transformational milestone for Arrowhead. This is a proud moment for all those involved in the discovery and development process and represents new hope for the estimated 6,500 people in the U.S. living with genetic or clinical FCS. This approval, and subsequent launch, marks the beginning of a new chapter in our journey—one rooted in our unwavering commitment to delivering life-changing therapies to patients with serious diseases," saidChristopher Anzalone, Ph.D., President and CEO at Arrowhead Pharmaceuticals. "REDEMPLO also represents the first FDA-approval for a medicine that leverages Arrowhead's proprietary and differentiated Targeted RNAi Molecule (TRiM™) platform. Arrowhead continues to lead the field in innovation, with the TRiM™ platform now potentially capable of delivering siRNA to seven different cell types in the body and the potential to simultaneously silence the expression of two genes in one molecule. The breadth of this technology with our growing commercial capabilities dramatically expands the diseases we can potentially address and the number of lives we can change."

The FDA approval was supported by clinical data from the Phase 3 PALISADE study, a randomized, double-blind, placebo-controlled trial in adults with clinically diagnosed or genetically confirmed FCS. The PALISADE study met its primary endpoint and all multiplicity-controlled key secondary endpoints, including demonstrating significant reductions in triglycerides and APOC3. In PALISADE, 25 mg REDEMPLO achieved deep and durable reductions in triglycerides, with a median change from baseline of -80% versus -17% in the pooled placebo group, and a lower numerical incidence of acute pancreatitis compared with placebo.

Lindsey Sutton Bryan, co-founder and co-president of the FCS Foundation added, "Today's approval marks a pivotal moment for people living with familial chylomicronemia syndrome and the physicians who support them. Because FCS symptoms are mostly invisible, this community historically has been often overlooked and misunderstood, making their journey to effective treatment especially difficult. We're grateful to Arrowhead for listening to patients and caregivers and incorporating their lived experiences into the development of this transformative therapy. Plozasiran offers real hope for a better future and shows what's possible when innovation is driven by empathy and collaboration addressing patients in need."

The most common adverse reactions in REDEMPLO treated patients (incidence ≥10% of patients treated with REDEMPLO and > 5% more frequently than with placebo) are hyperglycemia, headache, nausea, and injection site reaction. The US approved package insert contains no contraindications, warnings, or precautions associated with the use of REDEMPLO.

The efficacy and safety results from the PALISADE study were presented at the European Society of Cardiology (ESC) Congress 2024 and the American Heart Association Scientific Sessions 2024 (AHA24) and simultaneously published in The New England Journal of Medicine and Circulation, respectively. ESC, AHA24, and other plozasiran presentations may be accessed on the Events and Presentations page in the Investors section of the Arrowhead website.

Underscoring the company's commitment to improving patient outcomes, ensuring access for patients, and providing value to the health system, Arrowhead is developing best-in-class solutions for the FCS community. Arrowhead is launching Rely On REDEMPLO, a patient support program providing support services and resources for patients at each stage of the treatment journey with REDEMPLO, including financial assistance options for eligible patients.

REDEMPLO will be available in the U.S. before the end of the year.

REDEMPLO was granted Breakthrough Therapy Designation, Fast Track Designation, and Orphan Drug Designation by the FDA for the treatment of patients with FCS and was granted Orphan Medicinal Product Designation by the European Medicines Agency for the treatment of patients with FCS.

Webcast and Conference Call and Details

Arrowhead will host a conference call and webcast to discuss the REDEMPLO FDA approval today at 1:30 p.m. ET.

A live webcast may be accessed 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.

About FCS

Familial chylomicronemia syndrome (FCS) is a severe and rare disease leading to extremely high triglyceride (TG) levels, typically over 880 mg/dL. Such severe elevations can lead to various serious signs and symptoms including acute and potentially fatal pancreatitis, chronic abdominal pain, diabetes, hepatic steatosis, and cognitive issues. Currently, there are limited therapeutic options to adequately treat FCS.

About the PALISADE Phase 3 Study

The PALISADE study (NCT05089084) was a Phase 3 placebo-controlled study to evaluate the efficacy and safety of plozasiran in adults with genetically confirmed or clinically diagnosed FCS. The primary endpoint of the study was percent change from baseline in fasting TG versus placebo at Month 10. A total of 75 subjects distributed across 39 different sites in 18 countries were randomized to receive 25 mg plozasiran, 50 mg plozasiran, or matching placebo once every three months. Participants who completed the randomized period were eligible to continue in a 2-part extension period, where all participants receive plozasiran.

About REDEMPLO® (plozasiran)

REDEMPLO (plozasiran) is approved by the U.S. Food and Drug Administration as an adjunct to diet to reduce triglycerides for adults with Familial Chylomicronemia Syndrome (FCS). REDEMPLO is an siRNA therapeutic designed to suppress the production of apoC-III, a protein produced in the liver that raises triglyceride levels by slowing their breakdown and clearance. By targeting apoC-III with sustained silencing, REDEMPLO delivers significant reductions in triglyceride levels. REDEMPLO is the first and only siRNA FDA-approved treatment studied in both genetically confirmed and clinically diagnosed patients living with FCS.

For more information about REDEMPLO, visit Our Medicines.

IMPORTANT SAFETY INFORMATION

CONTRAINDICATIONS

None.

ADVERSE REACTIONS

Most common adverse reactions in REDEMPLO treated patients (incidence ≥10% of patients treated with REDEMPLO and >5% more frequently than with placebo) are hyperglycemia, headache, nausea, and injection site reaction.

Please see full Prescribing Information for REDEMPLO®.

About Plozasiran

Plozasiran is a first-in-class investigational RNA interference (RNAi) therapeutic designed to reduce production of apolipoprotein C-III (apoC-III) which is a component of triglyceride rich lipoproteins (TRLs) and a key regulator of triglyceride metabolism. ApoC-III 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 apoC-III, thereby reducing triglycerides and restoring lipids to more normal levels.

In addition to the FDA approval of REDEMPLO as an adjunct to diet to reduce triglycerides for adults with Familial Chylomicronemia Syndrome, plozasiran has been submitted to additional global regulatory authorities for review and marketing authorization. Plozasiran is also being investigated in the SHASTA-3, SHASTA-4, and SHASTA-5 Phase 3 studies in patients with severe hypertriglyceridemia and the MUIR Phase 3 study in patients with mixed hyperlipidemia.

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, 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 of REDEMPLO (plozasiran) 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 believes 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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