Arrowhead Pharmaceuticals Receives Positive CHMP Opinion Recommending Approval of REDEMPLO® (plozasiran) to Reduce Triglycerides in Adults with Familial Chylomicronemia Syndrome (FCS) in Europe

April 24, 2026

– If confirmed by the European Commission, REDEMPLO would be the first and only siRNA medicine authorized in the EU for both genetically confirmed and clinically diagnosed adult patients with 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 CHMP opinion is based on positive results from the Phase 3 PALISADE study where REDEMPLO reduced triglycerides by a median of 80% from baseline and significantly fewer cases of acute pancreatitis were seen in patients using REDEMPLO compared with those using placebo

PASADENA, Calif.--(BUSINESS WIRE)--Apr. 24, 2026-- Arrowhead Pharmaceuticals, Inc. (NASDAQ: ARWR) today announced that the European Medicines Agency's Committee for Medicinal Products for Human Use (EMA CHMP) has adopted a positive opinion, recommending the approval of REDEMPLO® (plozasiran), a small interfering RNA (siRNA) medicine, as an adjunct to diet to reduce triglyceride levels in adult patients with familial chylomicronemia syndrome (FCS). FCS remains widely underdiagnosed and affects an estimated 1 to 13 people per million globally. This rare disease is characterized by triglyceride levels that can be orders of magnitude higher than normal, leading to a substantially higher risk of developing acute, recurrent, and potentially fatal pancreatitis.

The European Commission is expected to issue a decision on REDEMPLO's Marketing Authorization in the second quarter of 2026. If approved, REDEMPLO will be the first and only siRNA medicine authorized in the European Union for both genetically confirmed and clinically diagnosed adult patients with FCS. This recommendation follows approvals in the United States, Canada, and China, as Arrowhead continues its efforts to increase global access to care for people living with FCS.

In a press release announcing the positive opinion, EMA stated, "Although other authorised medicines can help people with FCS confirmed by genetic testing, REDEMPLO does not require genetic confirmation of the condition, thus providing a treatment option for more adults with FCS and addressing the unmet medical need in these patients."

"We are pleased with the CHMP's recommendation, which brings us closer to potential approval of REDEMPLO as a new treatment option for people living with genetically or clinically confirmed FCS across Europe," said Christopher Anzalone, Ph.D., President and CEO at Arrowhead Pharmaceuticals. "This milestone reflects the strength of the clinical data and the ability of our TRiM™ platform to enable targeted siRNA therapies to potentially reach multiple tissues and disease areas. We believe REDEMPLO could represent an important new option for the FCS community in Europe, and we are working with regulators and the healthcare community to bring this therapy to more patients as quickly as possible."

Harnessing Arrowhead's proprietary Targeted RNAi Molecule (TRiM™) platform, REDEMPLO is designed to silence the mRNA encoding apolipoprotein C-III (apoC-III). ApoC-III is a key regulator of triglyceride metabolism that inhibits triglyceride catabolism and clearance, resulting in elevated triglyceride levels. Individuals with genetic loss-of-function variants in APOC3 typically have markedly lower triglyceride levels and a reduced risk of atherosclerotic cardiovascular disease.

The CHMP positive opinion 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 apoC-III and in the incidence of acute pancreatitis in the pooled dose groups. In PALISADE, 25 mg REDEMPLO reduced triglycerides by a median of 80% from baseline versus a 17% reduction with placebo and significantly fewer cases of acute pancreatitis were seen in patients using REDEMPLO compared with those using placebo. REDEMPLO is self-administered via subcutaneous injection once every three months.[1,2]

The most common adverse reactions are hyperglycaemia (12.8%), headache (6.8%), nausea (4.7%), and injection site reaction (4.7%).

"Patients with FCS face a substantial burden of disease and are at life-long risk of acute pancreatitis, with few effective treatment options available," said Gerald F Watts, DSc, PhD, MD, FRCP, Professor of Cardio-metabolic Medicine, University of Western Australia. "The results from the PALISADE study demonstrate that plozasiran can achieve substantial and sustained reductions in triglycerides in patients with FCS. These data highlight the potential of targeted RNA interference approaches in addressing conditions like FCS that have so far been difficult to treat. Plozasiran is a major advance in the care of FCS and a significant step toward expanding treatment options for patients across Europe."

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.

REDEMPLO has been granted Orphan Designation by the EMA for the treatment of patients with FCS, and Breakthrough Therapy Designation, Fast Track Designation, and Orphan Drug Designation by the U.S. FDA for the treatment of patients with FCS.

About FCS

Familial chylomicronemia syndrome (FCS) is a severe and rare disease leading to extremely high triglyceride (TG) levels, typically over 10 mmol/L (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 the first and only siRNA treatment approved in these countries that has been studied in both genetically confirmed and clinically diagnosed patients living with FCS. REDEMPLO is a first-in-class 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 self-administered via subcutaneous injection once every three months.

The EMA CHMP has adopted a positive opinion recommending the European Marketing Authorization of REDEMPLO (plozasiran), which is already approved by the U.S. Food and Drug Administration, Health Canada, and China's National Medical Products Administration as an adjunct to diet to reduce triglycerides for adults with Familial Chylomicronemia Syndrome (FCS).

In addition to approvals of REDEMPLO in the U.S., Canada, and China, plozasiran has been submitted to additional global regulatory authorities for review and marketing authorization. Plozasiran is also being investigated in the SHASTA-3 (NCT06347003), SHASTA-4 (NCT06347016), and SHASTA-5 (NCT06880770) Phase 3 studies in adults with severe hypertriglyceridemia and the MUIR-3 (NCT06347133) Phase 3 study in adults with hypertriglyceridemia. In December 2025, plozasiran was granted Breakthrough Therapy designation by the U.S. FDA in severe hypertriglyceridemia.

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.

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 candidates 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 if approved; 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, including the safety and efficacy of our products and product candidates, pricing and reimbursement decisions related to our products if approved, 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.

[1] Watts GF, Rosenson RS, Hegele RA, Goldberg IJ, Gallo A, Mertens A, Baass A, Zhou R, Muhsin M, Hellawell J, et al. Plozasiran for managing persistent chylomicronemia and pancreatitis risk. *N Engl J Med*. 2024;392:127–137. https://doi.org/10.1056/nejmoa2409368 PMID: 39225259

[2] Watts GF, Hegele RA, Rosenson RS et al. Temporal Effects of Plozasiran on Lipids and Lipoproteins in Persistent Chylomicronemia. Circulation. 2025:151(10); 733-736; https://doi.org/10.1161/CIRCULATIONAHA.124.072860 PMID:39549263

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