Arrowhead Pharmaceuticals Acquires FDA Priority Review Voucher

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- Voucher to be used to accelerate review of a planned sNDA for plozasiran in severe hypertriglyceridemia

PASADENA, Calif.--(BUSINESS WIRE)--Aug. 4, 2026-- Arrowhead Pharmaceuticals, Inc. (NASDAQ: ARWR) today announced that it has signed an agreement with an undisclosed third party to acquire a U.S. Food and Drug Administration (FDA) Rare Pediatric Disease Priority Review Voucher (PRV). The company intends to utilize the PRV for its upcoming Supplemental New Drug Application (sNDA) submission for plozasiran, anticipated before the end of 2026, to seek approval to expand the approved indication to include patients with severe hypertriglyceridemia (sHTG). SHTG is a condition that significantly increases the risk of acute pancreatitis, which is associated with repeated hospital admissions and can be fatal. A Priority Review designation accelerates FDA's goal to take action on an application to six months, compared to 10 months under standard review.

"The recent topline results for the global Phase 3 SHASTA-3 and SHASTA-4 clinical studies have strengthened our confidence in the potential for plozasiran to transform the treatment of patients with sHTG," said Christopher Anzalone, Ph.D., President and CEO at Arrowhead. "The acquisition of a priority review voucher potentially accelerates the process of bringing this important new medicine to patients."

Plozasiran has received regulatory approval, as REDEMPLO®, in the United States, the European Union, China, Australia, and Canada as an adjunct to diet to reduce triglycerides in adults with genetically confirmed or clinically diagnosed familial chylomicronemia syndrome (FCS), which is the most severe form of sHTG.

Closing of the transaction is subject to the expiration or termination of the waiting period under the Hart-Scott-Rodino Antitrust Improvements Act of 1976 and other customary conditions.

About Severe Hypertriglyceridemia

Severe hypertriglyceridemia (sHTG) is characterized by triglyceride (TG) levels greater than 500 mg/dL, with the most severe form being familial chylomicronemia syndrome (FCS) where TGs typically exceed 880 mg/dL. SHTG significantly increases the risk of acute pancreatitis (AP), which can often include recurrent attacks requiring repeat hospital admissions and worsening outcomes. AP risk is proportional to the number, characteristics, and concentration of triglyceride rich lipoproteins (TRLs), particularly chylomicrons, and increases as TGs rise. Elevated TGs can also increase the risk of atherosclerotic cardiovascular disease (ASCVD). Limited treatment options exist to sustainably reduce TGs below guideline directed risk thresholds.

About REDEMPLO® (plozasiran)

REDEMPLO (plozasiran) is currently approved by the U.S. Food and Drug Administration, Health Canada, China's National Medical Products Administration, the Australian Therapeutic Goods Administration, and by the European Commission as an adjunct to diet to reduce triglycerides for adults with FCS. REDEMPLO is the first and only siRNA treatment approved in these countries to be studied in both clinically diagnosed and genetically confirmed patients living with FCS.

REDEMPLO is designed to suppress the production of apolipoprotein C-III (APOC3), a protein produced in the liver that raises triglyceride levels by slowing their breakdown and clearance. By targeting APOC3 with sustained silencing, REDEMPLO delivers significant reductions in triglyceride levels. REDEMPLO is self-administered via subcutaneous injection once every three months.

REDEMPLO has been granted Orphan Medicinal Product 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 and was also granted Breakthrough Therapy designation by the U.S. FDA in severe hypertriglyceridemia.

Sanofi acquired the rights to develop and commercialize REDEMPLO in Greater China, with Arrowhead retaining rights to REDEMPLO in all geographies, outside of Greater China.

For more information about REDEMPLO, visit Our Medicines.

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 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 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.

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