Arrowhead Pharmaceuticals Files Complaint for Declaratory Judgment Against Ionis Pharmaceuticals

September 11, 2025

PASADENA, Calif.--(BUSINESS WIRE)--Sep. 11, 2025-- Arrowhead Pharmaceuticals, Inc. (NASDAQ: ARWR) today announced that yesterday it filed a Complaint for Declaratory Judgment in the United States District Court for the District of Delaware against Ionis Pharmaceuticals, Inc., to declare that Ionis's United States Patent No. 9,593,333 ("the '333 patent") is invalid and not infringed by Arrowhead's planned commercialization of investigational plozasiran, which is under FDA review. Arrowhead intends to vigorously pursue its claims in this litigation.

Ionis has threatened legal action against Arrowhead concerning its plozasiran product, including allegations of infringement of the '333 patent. Arrowhead believes those infringement allegations are baseless and is confident it will prove the '333 patent is invalid and not infringed.

"One of the most important values at Arrowhead is putting the needs of patients first. It is unfortunate and troubling that Ionis Pharmaceuticals is attempting to take action that clearly puts their corporate goals ahead of the needs of patients with familial chylomicronemia (FCS), a severe and rare disease characterized by extremely high triglyceride levels which can lead to acute and potentially fatal pancreatitis, chronic abdominal pain, diabetes, hepatic steatosis, and cognitive issues. Arrowhead will not tolerate efforts by Ionis to limit the availability of a potentially important new medicine to members of the FCS community," said Christopher Anzalone, President and CEO at Arrowhead. "Arrowhead has been an innovator in RNAi therapeutics for decades and has made countless important discoveries leading to the development of plozasiran and the proprietary TRiM™ platform. Arrowhead has multiple issued US patents that cover plozasiran for the treatment of patients with FCS based entirely on work developed internally at Arrowhead, which Ionis was not involved with and provided no contribution to whatsoever."

Arrowhead is not seeking monetary relief in this action, but rather a declaratory decree establishing that the '333 patent is invalid and/or not infringed by the manufacture, use, sale, or offer for sale of plozasiran.

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 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 that is proposed for marketing approval 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 completed PALISADE Phase 3 study in patients with FCS, the ongoing SHASTA studies in patients with SHTG, and the ongoing MUIR 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 Medicinal Product Designation by the European Medicines Agency. Investigational plozasiran has been submitted for marketing authorization in treatment of FCS to multiple global regulatory authorities but 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 report 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. These forward-looking statements include, but are not limited to, statements about our planned commercialization of investigational plozasiran and beliefs and expectations regarding the merit of the Ionis letter, the validity and enforceability of the '333 Patent, the Company's patent portfolio, and the Company's claims and lawsuit against the Defendant. These statements are based upon our current expectations and speak only as of the date hereof. Our 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 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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