



Ionis partner Novartis announces Lp(a)HORIZON Phase 3 topline results for pelacarsen in patients with elevated Lp(a) and established cardiovascular disease (CVD)

September 4, 2026

CARLSBAD, Calif.--(BUSINESS WIRE)--Sep. 4, 2026-- [Ionis Pharmaceuticals, Inc.](#) (Nasdaq: IONS) partner Novartis today announced that the pelacarsen Phase 3 Lp(a)HORIZON study, a pioneering cardiovascular outcomes trial, did not meet its primary endpoint of reducing the risk of cardiovascular events, a composite of cardiovascular death, non-fatal myocardial infarction, non-fatal stroke, and urgent coronary revascularization requiring hospitalization, compared to placebo. Consistent with previous studies, substantially lower lipoprotein (a) [Lp(a)] levels were achieved with pelacarsen. Elevated Lp(a) is an inherited cardiovascular risk factor affecting approximately one in five people worldwide with no approved targeted treatment.

"Although pelacarsen substantially lowered Lp(a) levels consistent with previous studies, we are disappointed that this did not translate to cardiovascular risk reduction," said Brett P. Monia, PhD, chief executive officer, Ionis. "Lp(a)HORIZON was the first pivotal Phase 3 trial to address the question as to whether lowering Lp(a) could reduce cardiovascular risk in people with established cardiovascular disease who are already receiving optimal cardiovascular care. While the study did not meet its primary endpoint, these results provide clarity that will meaningfully inform future cardiovascular care. Ionis continues to drive the independent commercialization of our wholly owned medicines, TRYNGOLZA®, DAWNZERO® and now ZANVASTRO™, and advance a robust pipeline of potentially transformational medicines."

Pelacarsen demonstrated an acceptable safety profile.

The full Lp(a)HORIZON data will be presented at an upcoming medical congress.

Ionis discovered and conducted the early development of pelacarsen. In February 2019, Novartis exercised an option to license the rights to develop and commercialize pelacarsen for targeted cardiovascular therapy.

About pelacarsen

Pelacarsen is an investigational antisense oligonucleotide (ASO) designed to potentially inhibit lipoprotein(a) [Lp(a)] production at its source.

About Elevated Lp(a)

Elevated lipoprotein (a) [Lp(a)] is an inherited, independent and causal cardiovascular disease (CVD) risk factor. Lp(a) levels are approximately 90% genetically determined and largely unaffected by diet or lifestyle. Lp(a) can contribute to plaque buildup and inflammation in the arteries, which can lead to plaque rupture and cardiovascular events. Approximately 20% of people worldwide, and nearly one-third of those with premature cardiovascular disease, have elevated Lp(a). U.S. and European guidelines recommend Lp(a) testing once in a lifetime for all adults.

About Lp(a)HORIZON

Lp(a)HORIZON (NCT04023552) is a Phase 3, randomized, placebo-controlled, double-blind, parallel-group, multi-center global cardiovascular outcomes trial evaluating the effect of pelacarsen on the incidence of 4-point major adverse cardiovascular events (MACE) in 8,323 patients with elevated Lp(a) and established CVD. The primary endpoint is a composite of cardiovascular death, non-fatal myocardial infarction, non-fatal stroke, and urgent coronary revascularization requiring hospitalization, evaluated in the overall population (Lp(a) ≥70 mg/dL) and subpopulation (Lp(a) ≥90 mg/dL). Study participants received guideline-directed treatments, including lipid lowering and antihypertensive therapies.

About Ionis Pharmaceuticals, Inc.

For more than three decades, Ionis has invented medicines that bring better futures to people with serious diseases. Ionis currently has marketed medicines and a leading pipeline in neurology, cardiometabolic disease and select areas of high patient need. As the pioneer in RNA-targeted medicines, Ionis continues to drive innovation in RNA therapies in addition to advancing new approaches in gene editing. A deep understanding of disease biology and industry-leading technology propels our work, coupled with a passion and urgency to deliver life-changing advances for patients. To learn more about Ionis, visit [ionis.com](#) and follow us on [X \(Twitter\)](#), [LinkedIn](#) and [Instagram](#).

Ionis Forward-looking Statements

This press release includes forward-looking statements regarding Ionis' business and the therapeutic and commercial potential of pelacarsen, our commercial medicines, additional medicines in development and technologies and our expectations regarding development and regulatory milestones. Any statement describing Ionis' goals, expectations, financial or other projections, intentions or beliefs is a forward-looking statement and should be considered an at-risk statement. Such statements are subject to certain risks and uncertainties including those inherent in the process of discovering, developing and commercializing medicines that are safe and effective for use as human therapeutics, and in the endeavor of building a business around such medicines. Ionis' forward-looking statements also involve assumptions that, if they never materialize or prove correct, could cause its results to differ materially from those expressed or implied by such forward-looking statements. Although Ionis' forward-looking statements reflect the good faith judgment of its management, these statements are based only on facts and factors currently known by Ionis. Except as required by law, we undertake no obligation to update any forward-looking statements for any reason. As a result, you are cautioned not to rely on these forward-looking statements. These and other risks concerning Ionis' programs are described in additional detail in Ionis' annual report on Form 10-K for the year ended December 31, 2025 and most recent Form 10-Q, which are on file with the Securities and Exchange Commission. Copies of these and other documents are available from the Company.

In this press release, unless the context requires otherwise, "Ionis," "Company," "we," "our" and "us" all refer to Ionis Pharmaceuticals and its subsidiaries.

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