

Ionis enters collaboration to advance next generation program targeting Lp(a) for cardiovascular disease

August 3, 2023

- *Builds on existing collaboration with Novartis for pelacarsen, which is currently being evaluated in a Phase 3 cardiovascular outcome study with data expected in 2025*
- *Ionis' new and advanced technologies will be used to create next generation compound targeting Lp(a)*
- *Ionis will receive a \$60 million upfront payment*

CARLSBAD, Calif., Aug. 3, 2023 /PRNewswire/ -- [Ionis Pharmaceuticals](#), Inc. (Nasdaq: IONS) today announced that it has entered a collaboration and license agreement with Novartis for the discovery, development and commercialization of a novel medicine for patients with lipoprotein(a), or Lp(a)-driven cardiovascular disease (CVD). This builds on the companies' existing collaboration focused on the development and commercialization of pelacarsen, which Novartis is currently evaluating in a Phase 3 cardiovascular outcome study. The next generation compound will be a potential follow-on to pelacarsen.

"We are pleased to expand our productive collaboration with Novartis aimed at delivering transformative therapies to patients with elevated Lp(a) who are at high risk of cardiovascular events," said Brett P. Monia, Ph.D., Ionis' chief executive officer. "This collaboration is designed to leverage Ionis' advancing RNA-targeting platform technologies to deliver a novel Lp(a)-targeting therapy that we expect will provide industry-leading efficacy and dosing frequency."

Ionis will receive a \$60 million upfront payment from Novartis and is eligible to earn development, regulatory and commercial milestone payments and tiered royalties. Novartis will be solely responsible for the development, manufacturing and potential commercialization of the next generation Lp(a) therapy.

The agreement is subject to the satisfaction of requirements of the Hart-Scott-Rodino Antitrust Improvements Act of 1976.

About Lp(a)

Lp(a) is a lipoprotein particle assembled in the liver that consists of a low-density lipoprotein cholesterol (LDL-C)-like particle and apolipoprotein(a). Lp(a) levels in the blood can vary greatly between individuals primarily due to genetic variations and do not correlate with LDL-C levels. Even patients with LDL-C lowered to target levels (<70 mg/dL) remain at high-risk of cardiovascular events if they have high levels of Lp(a). Elevated Lp(a) is a genetically determined condition that is not responsive to lifestyle changes, therefore patients are unable to adequately control their Lp(a) levels through improved diet or increased physical activity. Elevated Lp(a) is recognized as an independent, genetic cause of coronary artery disease, heart attack, stroke, peripheral arterial disease and aortic stenosis. Currently, there is no effective drug therapy to specifically and robustly lower elevated levels of Lp(a).

About Pelacarsen

Pelacarsen was discovered by Ionis and licensed to Novartis in 2019 for exclusive worldwide development, manufacturing and commercialization. It is an investigational antisense medicine designed to reduce apolipoprotein(a) production in the liver to offer a direct approach for reducing circulating Lp(a). It is estimated that more than eight million people worldwide have elevated Lp(a) and CVD.

Pelacarsen is currently being evaluated in Lp(a) HORIZON ([NCT04023552](#)), a global, multicenter, double-blind, placebo-controlled pivotal Phase 3 study conducted by Novartis. The study completed enrollment with 8,323 participants. Data are expected in 2025.

About Ionis Pharmaceuticals, Inc.

For more than 30 years, Ionis has been a leader in RNA-targeted therapy, pioneering new markets and changing standards of care. Ionis currently has four marketed medicines and a promising late-stage pipeline highlighted by cardiovascular and neurological franchises. Our scientific innovation began and continues with the knowledge that sick people depend on us, which fuels our vision to become the leader in genetic medicine, utilizing a multi-platform approach to discover, develop and deliver life-transforming therapies.

To learn more about Ionis visit www.ionispharma.com and follow us on Twitter @ionispharma.

Ionis' Forward-looking Statements

This press release includes forward-looking statements regarding Ionis' business and the therapeutic and commercial potential of pelacarsen, Ionis' next generation program targeting Lp(a), Ionis' technologies and other products in development. 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 related to our commercial products and the medicines in our pipeline, and particularly 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. 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 Dec. 31, 2022, and the most recent Form 10-Q quarterly filing, 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" refers to Ionis Pharmaceuticals and its subsidiaries.

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