

Ionis' pioneering antisense technology to be featured in multiple presentations at American Heart Association (AHA) Scientific Sessions 2020

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- Ionis' novel antisense medicine targeting PCSK9 demonstrates best-in-class potential for the treatment of patients with high cholesterol at risk for cardiovascular disease

CARLSBAD, Calif., Nov. 12, 2020 /PRNewswire/ -- Ionis Pharmaceuticals, Inc. (NASDAQ: IONS), announced today the upcoming presentations of clinical and non-clinical data for several of its investigational antisense medicines for cardiovascular disease at the American Heart Association (AHA) Scientific Sessions 2020, to be held virtually November 13-17, 2020.



Two of the abstracts accepted for presentation will provide new data from studies of ION449, also known as AZD8233 for subcutaneous administration and AZD6615 for oral administration. ION449 is an investigational antisense medicine targeting PCSK9, which is integrally involved in the regulation of LDL-cholesterol. ION449 is being developed as part of a collaboration between Ionis and the biopharmaceutical company AstraZeneca. Data presented include a study evaluating the safety, tolerability, pharmacokinetics and pharmacodynamics of single ascending subcutaneous doses of ION449 in patients with elevated LDL-cholesterol and a separate pre-clinical study evaluating this investigational medicine for once-daily oral dosing.

Additional presentations by Ionis scientists will include a review of RNA-targeted treatments designed to inhibit the production of apolipoprotein C-III (apoC-III) and lipoprotein(a) or Lp(a). The apoC-III protein is an independent cardiovascular risk factor that regulates triglyceride metabolism in the blood. Ionis' AKCEA-APOCIII-L_{Rx} is an investigational **L**igand-**C**onjugated **A**ntisense (LICA) medicine designed to inhibit the production of apoC-III for patients who are at risk for cardiometabolic disease due to elevated triglyceride levels. AKCEA-APOCIII-L_{Rx} is expected to start a Phase 3 study in patients with familial chylomicronemia syndrome (FCS) by year end. Elevated Lp(a) is an independent, genetic risk factor for cardiovascular disease that cannot be well controlled with lifestyle modifications such as diet or exercise or with treatment using existing lipid-lowering therapies. Ionis' pelacarsen is an investigational LICA medicine designed to reduce the production of apolipoprotein(a) in patients who are at risk for cardiometabolic disease due to high Lp(a). Pelacarsen is currently in a Phase 3 cardiovascular outcomes study.

Following are presentations of Ionis programs and collaborations:

Cardiovascular Seminars:

- Friday, November 13, 11:10 a.m. CST 'Experimental Therapy for APOCIII Lowering: ASO, siRNA,' Rosanne Crooke, Ph.D., senior strategic consultant at Ionis
- Friday, November 13, 11:20 a.m. CST 'Experimental Therapy for Lp(a) Lowering,' Sotirios "Sam" Tsimikas, M.D., Ph.D., senior vice president, clinical development and leader, cardiovascular franchise

Poster Presentations in Collaboration with AstraZeneca:

- Friday, November 13, 9:00 a.m. CST 'An Oral Antisense Oligonucleotide for PCSK9 Inhibition in Humans' (Poster #P244)
- Friday, November 13, 9:00 a.m. CST 'Single Dose Safety, Pharmacokinetics, and Pharmacodynamics of a Potent PCSK9 Synthesis Inhibitor, AZD8233, in Subjects With Elevated LDL Cholesterol' (Poster #MP515)

A full list of presentations can be found on the [AHA Scientific Sessions website](#).

About Ionis Pharmaceuticals, Inc.

As the leader in RNA-targeted drug discovery and development, Ionis has created an efficient, broadly applicable, drug discovery platform called antisense technology that can treat diseases where no other therapeutic approaches have proven effective. Our drug discovery platform has served as a springboard for actionable promise and realized hope for patients with unmet needs. We created the first and only approved treatment for all patients, children and adults with spinal muscular atrophy, as well as the world's first RNA-targeted therapeutic approved for the treatment of polyneuropathy in adults with hereditary transthyretin amyloidosis. Our sights are set on all the patients we have yet to reach with a pipeline of more than 40 novel medicines designed to potentially treat a broad range of disease, including neurological, cardio-renal, metabolic, infectious, and pulmonary diseases.

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

IONIS FORWARD-LOOKING STATEMENT

This press release includes forward-looking statements regarding Ionis' business, the therapeutic and commercial potential of our products, Ionis' technologies and 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, particularly those inherent in the process of discovering, developing and commercializing drugs that are safe and effective for use as human therapeutics, and in the endeavor of building a business around such drugs. 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 December 31, 2019, and the most recent Form 10-Q quarterly filing, which are on file with the SEC. 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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