Ionis and AstraZeneca close deal to develop and commercialize eplontersen

December 29, 2021

CARLSBAD, Calif., Dec. 29, 2021 /PRNewswire/ -- Ionis Pharmaceuticals, Inc. (Nasdaq: IONS), the leader in RNA-targeted therapies, today announced the closing of the collaboration agreement with AstraZeneca to develop and commercialize eplontersen, Ionis' investigational antisense medicine for the treatment of transthyretin amyloidosis (ATTR), following expiration of the waiting period under the Hart-Scott-Rodino Antitrust Improvements Act of 1976 (HSR Act).

Eplontersen, formerly known as IONIS-TTR-L_{Rx}, is designed to reduce the production of transthyretin, or TTR protein, to treat ATTR, a systemic, progressive and fatal disease. It uses lonis' advanced **Li**gand-**C**onjugated **A**ntisense (LICA) technology.

As previously announced, the companies will jointly develop and commercialize eplontersen in the U.S. AstraZeneca has an exclusive license for eplontersen outside the U.S., except certain countries in Latin America. Under the terms of the agreement, lonis will receive a \$200 million upfront payment, up to \$485 million in development and approval milestones, and up to \$2.9 billion in sales-related milestone payments. The collaboration includes territory-specific development, commercial and medical affairs cost-sharing provisions. Ionis is also eligible to earn royalties in the range of low double-digit to mid-20s percentage depending on region. Additional details about the agreement can be found in Ionis' Form 8-K filed with the Securities and Exchange Commission on Dec. 7, 2021.

About Ionis Pharmaceuticals, Inc.

For more than 30 years, lonis has been the leader in RNA-targeted therapy, pioneering new markets and changing standards of care with its novel antisense technology. Ionis currently has three marketed medicines and a premier late-stage pipeline highlighted by industry-leading neurological and cardiometabolic franchises. Our scientific innovation began and continues with the knowledge that sick people depend on us, which fuels our vision of becoming one of the most successful biotechnology companies.

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

Forward-looking Statements

This press release includes forward-looking statements regarding lonis' business and the therapeutic and commercial potential of lonis' technologies, eplontersen and other products in development. Any statement describing lonis' 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 the impact COVID-19 could have on our business, and including but not limited to 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 December 31, 2020, 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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