

Ionis licenses hepatitis B program to GSK

August 27, 2019

- GSK exercises option to license program following positive Phase 2 results - Transaction provides Ionis with significant participation in the commercial success of HBV program

CARLSBAD, Calif., Aug. 27, 2019 /PRNewswire/ -- Ionis Pharmaceuticals, Inc. (Nasdaq: IONS), the leader in RNA-targeted therapeutics, announced today that GSK exercised its option to license Ionis' antisense medicines for people with chronic hepatitis B virus (CHB) infection following positive Phase 2 results.

CHB is a chronic viral infection of the liver that can lead to significant and potentially fatal health conditions, including cirrhosis, liver failure and liver cancer. Current treatments for patients with CHB provide low levels of hepatitis B virus S antigen seroclearance, leaving many patients without a treatment to achieve sustained disease remission. There are more than 200 million patients worldwide living with CHB.

"By targeting the root cause of CHB-related liver disease, our antisense technology can potentially deliver a transformative medicine for people who suffer from chronic hepatitis B virus infection," said Brett P. Monia, chief operating officer at Ionis. "We believe GSK's development and commercialization expertise in infectious diseases makes them the ideal partner to help address this high unmet need."

As a part of the licensing agreement, Ionis is eligible to receive license fees and milestone payments up to \$262 million, including a \$25 million license fee. In addition, Ionis is also eligible to receive tiered royalties in the low double digits on net sales. GSK is now responsible for all development, regulatory and commercialization activities and costs.

This transaction is subject to clearances under the Hart-Scott Rodino Antitrust Improvements Act. Review of the full data package by Ionis and GSK is ongoing and the positive results from the Phase 2 program will be presented at an upcoming medical congress.

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 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 treat a broad range of diseases including cardiovascular diseases, neurological diseases, infectious diseases, pulmonary diseases and cancer.

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' alliance with GSK and the development, activity, therapeutic potential, commercial potential and safety of Ionis' antisense medicines for people with chronic hepatitis B virus infection, IONIS-HBV_{Rx} and IONIS-HBV-L_{Rx}. 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, 2018, and its 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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