

Ionis Pharmaceuticals Announces that GSK has Initiated a Phase 1 Study of IONIS-HBV-L Rx

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Ionis Earns \$1.5M Milestone Payment

CARLSBAD, Calif., Jan. 13, 2016 /PRNewswire/ -- Ionis Pharmaceuticals, Inc. (NASDAQ: IONS) today announced that GSK has initiated a Phase 1 study of IONIS-HBV-L_{Rx}. Ionis earned a \$1.5 million milestone payment from GSK related to the initiation of this study. IONIS-HBV-L_{Rx}, which was previously referred to as ISIS-GSK6-L_{Rx}, is a drug Ionis is developing with GSK for the treatment of patients with hepatitis B virus (HBV). IONIS-HBV-L_{Rx} incorporates Ionis' LIgand Conjugated Antisense (LICA) technology that increases drug potency by enhancing delivery of the drug to its target tissue, which for this drug is the liver. Ionis and GSK are also collaborating on the development of IONIS-HBV_{Rx}, a generation 2.0+ antisense HBV drug. Both IONIS-HBV_{Rx} and IONIS-HBV-L_{Rx} are part of Ionis' alliance with GSK



"HBV infection is a serious health problem that affects the liver and can lead to significant and potentially fatal health conditions, including cirrhosis, liver failure and liver cancer. Current treatments for patients with chronic HBV do not effectively clear HBV, and these patients are unable to fully control HBV infection and achieve sustained disease remission. As such, more effective HBV medicines that provide greater viral inhibition and permit greater immune system activity against the viral antigens are needed," said C. Frank Bennett, Ph.D., senior vice president, research at Ionis Pharmaceuticals.

"Together with GSK, we plan to make progress on both of our HBV medicines in 2016. GSK plans to initiate a Phase 2 study on IONIS-HBV_{Rx} in patients with HBV infection and to complete the Phase 1 study on IONIS-HBV-L_{Rx}. IONIS-HBV-L_{Rx} is our first anti-infective drug in development that incorporates our LICA technology, which, due to its potential for enhanced potency, may have the benefit of robust activity paired with infrequent very-low doses," concluded Dr. Bennett.

Dr Zhi Hong, Senior Vice President Infectious Diseases GSK, commented "We are pleased that the start of these early clinical studies marks further progress in our collaboration with Ionis. We look forward to developing such innovative treatments that may allow HBV patients to achieve sustained remission."

ABOUT IONIS PHARMACEUTICALS, INC.

Ionis is the leading company in RNA-targeted drug discovery and development focused on developing drugs for patients who have the highest unmet medical needs, such as those patients with severe and rare diseases. Using its proprietary antisense technology, Ionis has created a large pipeline of first-in-class and/or best-in-class drugs, with over a dozen drugs in mid- to late-stage development. Drugs currently in Phase 3 development include volanesorsen, a drug Ionis is developing and plans to commercialize through its wholly owned subsidiary, Akcea Therapeutics, to treat patients with familial chylomicronemia syndrome and familial partial lipodystrophy; IONIS-TTR_{Rx}, a drug Ionis is developing with GSK to treat patients with all forms of TTR amyloidosis; and nusinersen, a drug Ionis is developing with Biogen to treat infants and children with spinal muscular atrophy. Ionis' patents provide strong and extensive protection for its drugs and technology. Additional information about Ionis is available at www.ionispharma.com.

IONIS' FORWARD-LOOKING STATEMENT

This press release includes forward-looking statements regarding Ionis Pharmaceuticals' alliance with GSK, the discovery,

development, activity, therapeutic and commercial potential and safety of IONIS-HBV-L_{Rx} and IONIS-HBV_{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, 2014, and its most recent quarterly report on Form 10-Q, which are on file with the SEC. Copies of these and other documents are available from the Company.

In December, 2015, the Company changed its name from Isis Pharmaceuticals, Inc. to Ionis Pharmaceuticals, Inc. 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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D. Wade Walke, Ph.D., Vice President, Corporate Communications and Investor Relations, 760-603-2741, Amy Williford, Ph.D., Associate Director, Corporate Communications, 760-603-2772