

Ionis announces GSK has advanced bepirovirsen into Phase 3 development

February 1, 2023

- *Initiation of the Phase 3 program expands Ionis' late-stage pipeline to 7 medicines in 9 indications*

CARLSBAD, Calif., Feb. 1, 2023 /PRNewswire/ -- [Ionis Pharmaceuticals, Inc.](#) (Nasdaq: IONS) today announced that GSK has initiated two randomized, double-blind, placebo-controlled Phase 3 studies evaluating the safety and efficacy of bepirovirsen (formerly IONIS-HBV_{Rx}), an investigational antisense medicine for the treatment of patients with chronic hepatitis B infection (CHB).

"The initiation of this Phase 3 program is an important step closer to delivering novel medicines to HBV patients in need," said Richard S. Geary, Ph.D., Ionis' executive vice president and chief development officer. "Promising Phase 2b results, which included the possibility for functional cure, offered an early indication that bepirovirsen has potential to provide significant benefit to patients living with CHB, a serious chronic health problem."

Current first-line therapies for patients with CHB can inhibit viral replication but cannot clear the virus and are generally taken for life. Bepirovirsen is a potential first-in-class treatment for CHB, uniquely designed to simultaneously reduce hepatitis B virus (HBV) replication and suppress viral antigens in hopes of stimulating innate immunity to help attain functional cure for patients.

Ionis earned a \$15 million milestone payment from GSK associated with the progression of bepirovirsen into the Phase 3 clinical studies.

For more information on the Phase 3 clinical studies please visit [B-Well 1](#) and [B-Well 2](#).

About Hepatitis B Virus Infection

Hepatitis B virus infection is a serious health problem that can lead to significant and potentially fatal health conditions, including cirrhosis, liver failure and liver cancer. Chronic hepatitis B infection is caused by the hepatitis B virus and is a major global health concern, affecting nearly 300 million people worldwide.^{1,2} Chronic HBV infection is one of the most common persistent viral infections in the world. Currently available therapies, although effective in reducing circulating HBV DNA in the blood, do not efficiently inhibit HBV antigen production and secretion.

About Bepirovirsen

Bepirovirsen (formerly known as IONIS-HBV_{Rx}), also known as GSK3228836, is an investigational antisense medicine designed to reduce the production of viral proteins associated with hepatitis B virus (HBV) infection and replication, including hepatitis B surface antigen, which is present in both acute and chronic infections and is associated with a poor prognosis in patients with chronic HBV infection.

Bepirovirsen was discovered and jointly developed with GSK. GSK licensed bepirovirsen from Ionis in August 2019 under a collaborative development and licensing agreement.

About Ionis Pharmaceuticals, Inc.

For more than 30 years, Ionis 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 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 undisputed 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 Ionis' technologies, bepirovirsen 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 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 Dec. 31, 2021, 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.

Ionis Pharmaceuticals® is a trademark of Ionis Pharmaceuticals, Inc.

¹ Trepo C, et al. Hepatitis B virus infection. Lancet. 2014 Dec 6; 384(9959): 2053-63. [https://doi.org/10.1016/S0140-6736\(14\)60220-8](https://doi.org/10.1016/S0140-6736(14)60220-8)

² World Health Organization, <https://www.who.int/news-room/fact-sheets/detail/hepatitis-b>

 View original content to download multimedia: <https://www.prnewswire.com/news-releases/ionis-announces-gsk-has-advanced-bepirovirsen-into-phase-3-development-301735783.html>

SOURCE Ionis Pharmaceuticals, Inc.

Ionis Investor Contact: 760-603-2331, Ionis Media Contact: 760-603-4679