Ionis partner GSK announces positive topline results from B-Well 1 and B-Well 2 Phase 3 studies for bepirovirsen, a potential first-in-class medicine for chronic hepatitis B

January 7, 2026

– Primary endpoint met in both trials –
– Bepirovirsen demonstrated a statistically significant and clinically meaningful functional cure rate –
– Chronic hepatitis B (CHB) accounts for ~56% of liver cancer cases and affects more than 250 million people worldwide –
– Global regulatory filings planned from Q1 2026 –

CARLSBAD, Calif.--(BUSINESS WIRE)--Jan. 7, 2026-- Ionis Pharmaceuticals, Inc. (Nasdaq: IONS) partner GSK today announced positive results from two pivotal Phase 3 studies, B-Well 1 and B-Well 2, evaluating the safety and efficacy of bepirovirsen, an investigational antisense oligonucleotide (ASO) for the treatment of chronic hepatitis B (CHB). The studies included over 1,800 patients from 29 countries. GSK licensed bepirovirsen from Ionis and the two companies have collaborated on its development.

CHB is a major health challenge affecting over 250 million people worldwide and is the leading cause of liver cancer. The current standard of care, nucleos(t)ide analogues, often requires lifelong therapy and the functional cure rates remain low, typically only 1%. Functional cure for CHB is when the virus can no longer be detected in the blood as measured by the sustained loss of hepatitis B surface antigen (HBsAg), a viral protein that signals ongoing infection, and undetectable hepatitis B virus DNA for at least 24 weeks after a finite course of treatment. Functional cure is associated with significant reduction in the risk of long-term liver complications, including liver cancer, as well as all-cause mortality.

The B-Well studies met their primary endpoint, and bepirovirsen demonstrated a statistically significant and clinically meaningful functional cure rate. Functional cure rates were significantly higher with bepirovirsen plus standard of care compared with standard of care alone. Results were statistically significant across all ranked endpoints, including in patients with baseline surface antigen (HBsAg) ≤1000 IU/ml where an even greater effect was demonstrated. The studies demonstrated an acceptable safety and tolerability profile consistent with what was reported in other studies.

“CHB is one of the most common, persistent viral infections in the world, with currently no approved therapy that can achieve meaningful functional cure. Bepirovirsen is uniquely positioned to effectively treat CHB based on its potential to reduce the replication of hepatitis B virus, suppress hepatitis B surface antigen and stimulate the immune system,” said Brett P. Monia, Ph.D., chief executive officer, Ionis. “Today’s positive results are made possible by the strategic collaboration between Ionis and GSK, and demonstrate that bepirovirsen has the potential to bring hope to the millions of people living with CHB. This is the first of five anticipated Phase 3 readouts from Ionis’ partnered programs this year, underscoring the broad applicability of our technology.”

Full results will be submitted for presentation at an upcoming scientific congress, published in a peer-reviewed journal and used to support regulatory submissions to health authorities worldwide. If approved, bepirovirsen has the potential to become the first finite, six-month therapeutic option for CHB and to serve as a backbone for future sequential treatment strategies.

Bepirovirsen has been recognized by global regulatory authorities for its innovation and potential to address significant unmet need in hepatitis B with Fast Track designation from the US FDA, Breakthrough Therapy designation in China and SENKU designation in Japan.

GSK licensed bepirovirsen from Ionis in 2019 under a collaborative development and licensing agreement. Under the terms of the agreement, Ionis received an upfront payment, license fee and development milestone payments and is eligible to receive an additional $150 million in regulatory and sales milestone payments as well as tiered royalties of 10-12% on net sales of bepirovirsen.

About B-Well 1 and B-Well 2

B-Well 1 (NCT05630807) and B-Well 2 (NCT05630820) trials are global multi-center, randomized, double-blind, placebo-controlled trials conducted in 29 countries. They assessed the efficacy, safety, pharmacokinetic profile, and the durability of functional cure in nucleos(t)ide analogue (NA)-treated participants with chronic hepatitis B and baseline surface antigen (HBsAg) ≤3000 IU/ml. The primary endpoint assessed the proportion of participants achieving functional cure in patients with baseline surface antigen (HBsAg) ≤3000 IU/ml. A key secondary endpoint evaluated functional cure in participants with baseline HBsAg ≤1000 IU/ml. Functional cure is defined as hepatitis B surface antigen (HBsAg) loss and undetectable HBV DNA for at least 24 weeks after a finite course of treatment.

About Chronic Hepatitis B (CHB)

Hepatitis B is a viral infection that can cause both acute and chronic liver disease. Chronic hepatitis B occurs when the immune system is unable to clear the virus, resulting in long-lasting infection that affects more than 250 million people worldwide. The disease causes approximately 1.1 million deaths each year, and accounts for approximately 56% of liver cancer cases globally. Many patients often require lifelong antiviral therapy for viral suppression; making functional cure a critical goal in disease management.

About Bepirovirsen

Bepirovirsen is an investigational antisense oligonucleotide (ASO) designed to recognize and orchestrate the destruction of the genetic components (i.e. RNA) of the hepatitis B virus that can lead to chronic disease, potentially allowing a person's immune system to regain control. Bepirovirsen inhibits the replication of viral DNA in the body, suppresses the level of hepatitis B surface antigen (HBsAg) in the blood, and stimulates the immune system to increase the chances of a durable and sustained response.

About Ionis Pharmaceuticals, Inc.

For three decades, Ionis has invented medicines that bring better futures to people with serious diseases. Ionis has marketed medicines and a leading pipeline in neurology, cardiometabolic and other areas of high patient need. As the pioneer in RNA-targeted medicines, Ionis continues to drive innovation in RNA therapies in addition to advancing new approaches in gene editing. A deep understanding of disease biology and industry-leading technology propels our work, coupled with a passion and urgency to deliver life-changing advances for patients. To learn more about Ionis, visit Ionis.com and follow us on X (Twitter), LinkedIn and Instagram.

Forward-looking Statements

This press release includes forward-looking statements regarding Ionis' business and the therapeutic and commercial potential of bepirovirsen, our commercial medicines, additional medicines in development and technologies. 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 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. Except as required by law, we undertake no obligation to update any forward-looking statements for any reason. 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, 2024, and most recent Form 10-Q, 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" all refer to Ionis Pharmaceuticals and its subsidiaries.

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