# Ionis partner GSK announces bepirovirsen accepted for Priority Review and granted Breakthrough Therapy Designation by U.S. FDA as a potential first-in-class medicine for chronic hepatitis B

April 28, 2026

*– Regulatory submission supported by Phase 3 B-Well trials demonstrating statistically significant and clinically meaningful functional cure rates in chronic hepatitis B -*

*– Breakthrough Therapy designation added to Fast Track Designation, recognizing potential for substantial improvement over existing treatments –*

*– Chronic hepatitis B is a leading cause of liver cancer globally –*

*– PDUFA Date of October 26, 2026 –*

CARLSBAD, Calif.--(BUSINESS WIRE)--Apr. 28, 2026-- Ionis Pharmaceuticals, Inc. (Nasdaq: IONS) partner GSK today announced the U.S. Food and Drug Administration (FDA) has accepted for Priority Review the New Drug Application (NDA) for bepirovirsen, an investigational antisense oligonucleotide (ASO) for the treatment of adults with chronic hepatitis B (CHB). The FDA has granted bepirovirsen Breakthrough Therapy designation and set a Prescription Drug User Fee Act (PDUFA) target action date of October 26, 2026.

“Over one million people in the U.S. are living with chronic hepatitis B and as of today, require lifelong antiviral therapy to suppress the virus,” said Brett P. Monia, Ph.D., chief executive officer, Ionis. “As the first medicine to deliver clinically meaningful functional cure rates, 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. This milestone reflects the broad impact of Ionis’ science, and we look forward to the potential for bepirovirsen to help millions of people living with this devastating condition around the world.”

Breakthrough Therapy designation is intended to expedite the review of medicines that treat a serious or life-threatening condition and have shown preliminary clinical evidence indicating the potential for substantial improvement over available therapies. The designation enables greater FDA guidance on a medicine’s development program. Priority Review designation is granted to marketing applications for medicines that, if approved, would provide a significant improvement in the safety or effectiveness of the treatment, prevention or diagnosis of a serious condition, with the expectation of the FDA taking action within six months, compared to 10 months under standard review. Bepirovirsen also received Fast Track designation from the U.S. FDA in February 2024.

CHB is a major public health challenge, affecting over 250 million people worldwide and 1.7 million in the U.S. The current standard of care, typically nucleos(t)ide analogues, often requires lifelong therapy and the functional cure rates remain low, typically only 1%. Functional cure occurs when hepatitis B virus DNA and viral protein, hepatitis B surface antigen (HBsAg), are undetectable in the blood for at least 24 weeks after stopping all treatment, indicating that the disease can be controlled by the immune system without medication. Achieving functional cure is associated with significant reduction in the risk of long-term liver complications, including liver cancer.

The regulatory submission and Breakthrough Therapy designation were supported by positive results from the Phase 3 B-Well 1 and B-Well 2 trials, where bepirovirsen demonstrated statistically significant and clinically meaningful functional cure rates. Functional cure rates were significantly higher with bepirovirsen plus standard of care compared to standard of care alone across all ranked endpoints, including in patients with lower baseline HBsAg levels, where an even greater effect was observed. Bepirovirsen demonstrated an acceptable safety and tolerability profile consistent with previous studies. The data will be presented at the European Association for the Study of the Liver (EASL) 2026 Congress in May and submitted for simultaneous publication in scientific peer-reviewed journal in 2026.

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

Bepirovirsen is currently under regulatory review with the European Medicines Agency (EMA), the China National Medical Products Administration (NMPA) and Japan’s Ministry of Health, Labour and Welfare (MHLW). Bepirovirsen was also awarded

Breakthrough Therapy Designation in China and SENKU Designation in Japan.

**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 HBsAg ≤3000 IU/ml. A key secondary endpoint evaluated functional cure in participants with baseline HBsAg ≤1000 IU/ml. Functional cure is defined as HBsAg being undetectable in the blood for at least 24 weeks after stopping all treatment, indicating that the disease is controlled by the immune system without medication.

**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 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 inhibit the production 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 reduces the production of RNA and viral proteins associated with HBV, 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.

**Ionis 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 and our expectations regarding development and regulatory milestones. 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, 2025, 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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