Precision BioSciences Receives U.S. FDA Fast Track Designation for PBGENE-HBV, a First-In-Class Gene Editing Therapy Designed to Eliminate the Root Cause of Chronic Hepatitis B

April 15, 2025 at 7:01 AM EDT

DURHAM, N.C.--(BUSINESS WIRE)--Apr. 15, 2025-- Precision BioSciences, Inc. (Nasdaq: DTIL), a clinical stage gene editing company utilizing its novel proprietary ARCUS® platform to develop *in vivo* gene editing therapies for diseases with high unmet need, today announced that the U.S. Food and Drug Administration (FDA) has granted Fast Track designation for PBGENE-HBV, the Company's lead wholly owned *in* vivo gene editing program designed to cure chronic hepatitis B by eliminating cccDNA, the key source of replicating hepatitis B virus (HBV), and inactivating integrated HBV DNA in hepatocytes.

"We are pleased to receive Fast Track designation from the FDA for PBGENE-HBV and believe this classification underscores the urgent need for improved treatment options for patients living with chronic hepatitis B," said Michael Amoroso, President and Chief Executive Officer at Precision BioSciences. "We've been encouraged by the initial safety and antiviral activity we have observed in the ELIMINATE-B trial and look forward to continuing to work closely with the FDA as we progress PBGENE-HBV through clinical development."

Precision is evaluating PBGENE-HBV in the ongoing global Phase 1 ELIMINATE-B trial, with clinical investigation in the United States, Moldova, Hong Kong, New Zealand, and the United Kingdom. The company anticipates sharing updates on the full low-dose cohort, including multiple dose administrations, and data from higher dose levels throughout 2025.

Fast Track designation is designed to facilitate development and expedite the review of drugs that are intended to treat serious or life-threatening conditions and address an unmet medical need. A drug that has received Fast Track designation may be eligible for more frequent meetings and communications with the FDA and rolling review of any application for marketing approval. A drug receiving Fast Track designation may also be eligible for Priority Review if relevant criteria are met.

About PBGENE-HBV (Viral Elimination Program):

PBGENE-HBV is Precision's wholly owned *in vivo* gene editing program under investigation in a global first-in-human clinical trial, which is designed to potentially cure chronic hepatitis B infection. Currently, it is estimated that 300 million people worldwide are afflicted with chronic hepatitis B. PBGENE-HBV is the first and only potentially curative gene editing program to enter clinical investigation that is specifically designed to eliminate cccDNA and inactivate integrated HBV DNA. Lipid nanoparticle technology for PBGENE-HBV has been provided by Acuitas Therapeutics Inc.

About Hepatitis B:

Hepatitis B is a leading cause of morbidity in the US and death globally, with no curative options currently available for patients. Despite the availability of approved antiviral therapies, an estimated 300 million people globally and 1-2 million people in the US are estimated to have chronic hepatitis B infection. An estimated 15% to 40% of patients with HBV infections may develop complications, such as cirrhosis, liver failure, or liver cancer (hepatocellular carcinoma), which account for the majority of HBV-related deaths.

Chronic hepatitis B infection is primarily driven by persistence of HBV cccDNA and integration of HBV DNA into the human genome in liver cells, the primary source of hepatitis B surface antigen (HBsAg) in late-stage disease. Current treatments for patients with HBV infection include agents that result in long-term viral suppression as indicated by reduction of circulating HBV DNA, but these therapies do not eradicate HBV cccDNA, rarely lead to functional cure, and require lifelong administration.

About Precision BioSciences, Inc.

Precision BioSciences, Inc. is a clinical stage gene editing company dedicated to improving life (DTIL) with its novel and proprietary ARCUS® genome editing platform that differs from other technologies in the way it cuts, its smaller size, and its simpler structure. Key capabilities and differentiating characteristics may enable ARCUS nucleases to drive more intended, defined therapeutic outcomes. Using ARCUS, the Company's pipeline is comprised of *in vivo* gene editing candidates designed to deliver lasting cures for the broadest range of genetic and infectious diseases where no adequate treatments exist. For more information about Precision BioSciences, please visit www.precisionbiosciences.com.

The ARCUS® platform is being used to develop *in vivo* gene editing therapies for sophisticated gene edits, including gene insertion (inserting DNA into gene to cause expression/add function), elimination (removing a genome e.g. viral DNA or mutant mitochondrial DNA), and excision (removing a large portion of a defective gene by delivering two ARCUS nucleases in a single AAV).

Forward-Looking Statements

This press release contains forward-looking statements within the meaning of the Private Securities Litigation Reform Act of 1995. All statements contained in this press release that do not relate to matters of historical fact should be considered forward-looking statements, including, without limitation, statements regarding the clinical development and expected safety, efficacy and benefit of PBGENE-HBV and our gene editing approaches including editing efficiency, and the suitability of ARCUS nucleases for gene insertion, gene elimination and gene excision and differentiation from other gene editing approaches; the expected timing of regulatory processes and clinical operations, including filings, studies, enrollment and clinical

data for PBGENE-HBV; the design of PBGENE-HBV to directly eliminate cccDNA and inactivate integrated HBV DNA with high specificity, potentially leading to functional cures; the encouraging initial safety and antiviral activity observed in the ELIMINATE-B clinical trial; plans to provide ongoing updates on the full low-dose cohort for the PBGENE-HBV study, including multiple dose administrations, and data from higher dose levels throughout 2025; and anticipated timing of clinical data . In some cases, you can identify forward-looking statements by terms such as “aim,” “anticipate,” “appear,” “approach,” “believe,” “contemplate,” “could,” “designed,” “encouraged”, “estimate,” “expect,” “goal,” “intend,” “look,” “may,” “mission,” “plan,” “possible,” “potential,” “predict,” “project,” “pursue,” “should,” “strive,” “suggest,” “target,” “will,” “would,” or the negative thereof and similar words and expressions.

Forward-looking statements are based on management’s current expectations, beliefs and assumptions and on information currently available to us. These statements are neither promises nor guarantees, and involve a number of known and unknown risks, uncertainties and assumptions, and actual results may differ materially from those expressed or implied in the forward-looking statements due to various important factors, including, but not limited to, our ability to become profitable; our ability to procure sufficient funding to advance our programs; risks associated with our capital requirements, anticipated cash runway, requirements under our current debt instruments and effects of restrictions thereunder, including our ability to raise additional capital due to market conditions and/or our market capitalization; our operating expenses and our ability to predict what those expenses will be; our limited operating history; the progression and success of our programs and product candidates in which we expend our resources; our limited ability or inability to assess the safety and efficacy of our product candidates; the risk that other genome-editing technologies may provide significant advantages over our ARCUS technology; our dependence on our ARCUS technology; the initiation, cost, timing, progress, achievement of milestones and results of research and development activities and preclinical and clinical studies, including clinical trial and investigational new drug applications; public perception about genome editing technology and its applications; competition in the genome editing, biopharmaceutical, and biotechnology fields; our or our collaborators’ or other licensees’ ability to identify, develop and commercialize product candidates; pending and potential product liability lawsuits and penalties against us or our collaborators or other licensees related to our technology and our product candidates; the U.S. and foreign regulatory landscape applicable to our and our collaborators’ or other licensees’ development of product candidates; our or our collaborators’ or other licensees’ ability to advance product candidates into, and successfully design, implement and complete, clinical trials; potential manufacturing problems associated with the development or commercialization of any of our product candidates; delays or difficulties in our and our collaborators’ and other licensees’ ability to enroll patients; changes in interim “top-line” and initial data that we announce or publish; if our product candidates do not work as intended or cause undesirable side effects; risks associated with applicable healthcare, data protection, privacy and security regulations and our compliance therewith; our or our licensees’ ability to obtain orphan drug designation or fast track designation for our product candidates or to realize the expected benefits of these designations; our or our collaborators’ or other licensees’ ability to obtain and maintain regulatory approval of our product candidates, and any related restrictions, limitations and/or warnings in the label of an approved product candidate; the rate and degree of market acceptance of any of our product candidates; our ability to effectively manage the growth of our operations; our ability to attract, retain, and motivate executives and personnel; effects of system failures and security breaches; insurance expenses and exposure to uninsured liabilities; effects of tax rules; effects of any pandemic, epidemic, or outbreak of an infectious disease; the success of our existing collaboration and other license agreements, and our ability to enter into new collaboration arrangements; our current and future relationships with and reliance on third parties including suppliers and manufacturers; our ability to obtain and maintain intellectual property protection for our technology and any of our product candidates; potential litigation relating to infringement or misappropriation of intellectual property rights; effects of natural and manmade disasters, public health emergencies and other natural catastrophic events; effects of sustained inflation, supply chain disruptions and major central bank policy actions; market and economic conditions; risks related to ownership of our common stock, including fluctuations in our stock price; our ability to meet the requirements of and maintain listing of our common stock on Nasdaq or other public stock exchanges; and other important factors discussed under the caption “Risk Factors” in our Annual Report on Form 10-K for the year ended December 31, 2024, as any such factors may be updated from time to time in our other filings with the SEC, which are accessible on the SEC’s website at www.sec.gov and the Investors page of our website under SEC Filings at investor.precisionbiosciences.com.

All forward-looking statements speak only as of the date of this press release and, except as required by applicable law, we have no obligation to update or revise any forward-looking statements contained herein, whether as a result of any new information, future events, changed circumstances or otherwise.

View source version on businesswire.com: https://www.businesswire.com/news/home/20250415414433/en/

Investor and Media Contact:
Naresh Tanna
Vice President of Investor Relations
naresh.tanna@precisionbiosciences.com

Source: Precision BioSciences, Inc.