Precision BioSciences Announces Oral Presentation of Positive ARCUS® Gene Editing Data at International Liver Congress 2023

June 22, 2023 at 7:00 AM EDT

- High antiviral activity with substantial and durable reductions in hepatitis B serum antigen and covalently closed circular DNA in preclinical studies of PBGENE-HBV

- Supports continued PBGENE-HBV development and planned submission of CTA/IND application

DURHAM, N.C.--(BUSINESS WIRE)--Jun. 22, 2023-- Precision BioSciences, Inc. (Nasdaq: DTIL), a clinical stage gene editing company developing ARCUS®-based *in vivo* gene editing and *ex vivo* allogeneic CAR T therapies, today announced that the company will present positive preclinical data from its PBGENE-HBV development program for the treatment of patients with chronic hepatitis B during an oral presentation (Abstract #OS-034) Thursday, June 22, 2023 at the European Association for Study of the Liver (EASL) Congress 2023 in Vienna, Austria.

Hepatitis B virus (HBV) causes inflammation and damage to the liver, leading to chronic infection and increased risk of death from liver cancer or cirrhosis. There is no cure for chronic hepatitis B and current treatments rarely result in functional cure, primarily due to persistence of viral DNA in the liver. In patients with chronic HBV, genetic material of the virus is converted within infected liver cells into covalently closed circular DNA (cccDNA) that acts as a template to make HBV copies. HBV also inserts its DNA into the human genome of infected liver cells. This integrated HBV DNA produces the viral protein, hepatitis B surface antigen (HBsAg), which is secreted in the blood. Presence of HBsAg is associated with poorer outcomes and elimination of HBsAg is necessary for functional cure of chronic hepatitis B. Using ARCUS, company scientists generated a highly specific and effective nuclease to eradicate chronic HBV infection. PBGENE-HBV is designed to inactivate cccDNA with direct cuts and edits as well as to inactivate integrated HBV DNA leading to long lasting reductions in HBsAg.

In an episomal adeno-associated virus (AAV) mouse model, company researchers demonstrated that administration of lipid nanoparticles containing mRNA encoding an HBV-targeted ARCUS nuclease resulted in a 96% reduction in serum HBsAg. In a follow-on experiment, treatment of HBV-infected primary human hepatocytes with the HBV-targeted ARCUS nuclease resulted in a 90% reduction of covalently closed circular DNA (cccDNA) and high specificity.

“Even with lifelong administration, the current treatments of chronic HBV rarely achieve a functional cure because they do not eradicate the cccDNA or inactivate the viral DNA that is integrated into the human genome in hepatocytes. Eliminating both the cccDNA and HBsAg are key parameters for achieving a functional cure of HBV,” said Jeff Smith, Co-Founder and Chief Research Officer at Precision Biosciences. “In preclinical models presented at EASL, we’ve demonstrated that administration of HBV-targeted ARCUS nucleases can achieve a near complete reduction in both HBsAg and cccDNA.”

These data continue to highlight the potential of the ARCUS gene editing platform to achieve a functional cure for patients and support continued development of PBGENE-HBV with planned submission of a CTA/IND application.

Presentation Details:

Title: A Gene Editing Approach for Chronic Hepatitis B: Elimination of Hepatitis B Virus In Vivo by Targeting cccDNA and Integrated Viral Genomes with a Sequence-Specific ARCUS Nuclease
Authors: Gorsuch C, et al.
Date and Time: Thursday, June 22, 2023, 6:15-6:30pm CEST / 12:15-12:30pm EDT
Abstract Number: OS-034
Session Title: Abstract session - Viral hepatitis B/D - New treatments
Session Room: Strauss 2-3

About Hepatitis B and the PBGENE-HBV development candidate:

Hepatitis B is a leading cause of morbidity in the US and death globally, with no curative options currently available for patients. In 2019, despite the availability of approved antiviral therapies, an estimated 300 million people globally and more than 1 million people in the US were 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 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. PBGENE-HBV is a highly specific, novel therapeutic approach to treating patients with chronic HBV infection. It’s designed to directly cut and edit cccDNA and inactivate integrated HBV DNA with high specificity, resulting in degraded cccDNA and a reduction in HBsAg.

About Precision BioSciences, Inc.

Precision BioSciences, Inc. is a clinical stage biotechnology company dedicated to improving life (DTIL) with its novel and proprietary ARCUS® genome editing platform. ARCUS is a highly precise and versatile genome editing platform designed with therapeutic safety, delivery, and control in mind. Using ARCUS, the Company's pipeline consists of several *in vivo* gene editing candidates designed to cure genetic and infectious diseases where no adequate treatments exist and multiple *ex vivo* clinical candidates. For more information about Precision BioSciences, please visit www.precisionbiosciences.com.

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 expected safety, efficacy, and benefit of our gene editing approaches including editing efficiency and delivery methods, the suitability of ARCUS nucleases for gene excision, the clinical development, nomination, and goals of our PBGENE-HBV program, the timing of the submission of a CTA/IND application for PBGENE-HBV, and therapeutic potential of an ARCUS gene editing approach for the treatment of chronic hepatitis B. In some cases, you can identify forward-looking statements by terms such as "aim," "anticipate," "approach," "believe," "contemplate," "could," "estimate," "expect," "goal," "intend," "look," "may," "mission," "plan," "possible," "potential," "predict," "project," "pursue," "should," "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, but involve 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 and requirements under our current debt instruments and effects of restrictions thereunder; risks associated with raising additional capital; our operating expenses and our ability to predict what those expenses will be; our limited operating history; the 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; public perception about genome editing technology and its applications; competition in the genome editing, biopharmaceutical, and biotechnology fields; our or our collaborators' ability to identify, develop and commercialize product candidates; pending and potential product liability lawsuits and penalties against us or our collaborators related to our technology and our product candidates; the U.S. and foreign regulatory landscape applicable to our and our collaborators' development of product candidates; our or our collaborators' ability to advance product candidates into, and successfully design, implement and complete, clinical or field trials; potential manufacturing problems associated with the development or commercialization of any of our product candidates; our ability to obtain an adequate supply of T cells from qualified donors; our ability to achieve our anticipated operating efficiencies at our manufacturing facility; delays or difficulties in our and our collaborators' 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 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' 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 the COVID-19 pandemic and variants thereof, or any pandemic, epidemic, or outbreak of an infectious disease; the success of our existing collaboration 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, and other important factors discussed under the caption "Risk Factors" in our Quarterly Report on Form 10-Q for the quarterly period ended March 31, 2023, 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.

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