Precision BioSciences Presents New Preclinical Safety Data for PBGENE-HBV Clinical Candidate at the European Association for the Study of the Liver Congress

June 5, 2024 at 7:01 AM EDT

- PBGENE-HBV specifically cuts HBV DNA without impacting the human genome

- PBGENE-HBV was well tolerated across multiple administrations with no off-target editing observed

- Preclinical safety data supports advancement of PBGENE-HBV to clinical trials as a potentially curative, finite treatment for chronic hepatitis B with IND and/or CTA on-track for submission in 2024

DURHAM, N.C.--(BUSINESS WIRE)--Jun. 5, 2024-- Precision BioSciences, Inc. (Nasdaq: DTIL), an advanced gene editing company utilizing its novel proprietary ARCUS® platform to develop in vivo gene editing therapies, today announced that the company will present preclinical data for its PBGENE-HBV clinical candidate at the European Association for the Study of the Liver Congress (EASL), highlighting the differentiated ability of ARCUS to make efficient, durable, and targeted elimination edits. The poster presentation highlights data in primary human hepatocytes demonstrating the high specificity and lack of detectable off-target editing for PBGENE-HBV at therapeutically relevant doses. The poster will also showcase non-human primate data demonstrating good tolerability of a multi-dosing approach for the treatment of chronic hepatitis B.

“We are pleased to present new safety data at EASL for our lead PBGENE-HBV program. These data highlight our ability to specifically target the HBV viral genome, with no detectable off-target editing in the human genome at relevant doses. In addition, the new non-human primate data shows that multiple administrations of PBGENE-HBV are well tolerated, enabling us to multi-dose in clinic,” said Jeff Smith, PhD, Chief Research Officer of Precision BioSciences. “Our work to date continues to highlight the potential of PBGENE-HBV as a curative treatment for chronic hepatitis B and further validates the capabilities of our proprietary ARCUS® platform to deliver safe and efficacious *in vivo* gene editing therapies. Looking ahead, we are well positioned to progress PBGENE-HBV, our first wholly owned ARCUS program into the clinic with an Investigational New Drug Application (IND) and/or Clinical Trial Application (CTA) in the 2024.”

The data to be presented highlights that PBGENE-HBV specifically cuts HBV DNA leading to elimination of cccDNA and inactivation of integrated HBV DNA without impacting any sites in the human genome, including no editing-associated translocations in HBV infected primary human hepatocytes. In addition, PBGENE-HBV was well-tolerated in non-human primates across multiple dose administrations, with only minor and transient elevations in liver transaminases that are normalized within 2 weeks and non-adverse changes in blood parameters. Preclinical safety data supports the advancement of PBGENE-HBV to clinical trials as a potentially curative treatment for chronic hepatitis B.

Full poster details are included below:

Title: Preclinical safety data for PBGENE-HBV gene editing program supports advancement to clinical trials as a potentially curative treatment for chronic hepatitis B
Abstract Number: LB195
Presenter: Emily Harrison, PhD, Senior Scientist - Hepatitis Research Leader, Precision BioSciences
Date and Time: Wednesday, June 5, 2024, 8:30 AM – Saturday, June 8, 2024, 5:00 PM CEST

About Hepatitis B and PBGENE-HBV:

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 eliminate cccDNA and inactivate integrated HBV DNA with high specificity, potentially leading to functional cures.

About Precision BioSciences, Inc.

Precision BioSciences, Inc. is an advanced 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 our product candidates and gene editing approaches including editing efficiency; the design of PBGENE-HBV to directly eliminate cccDNA and inactivate integrated HBV DNA with high specificity, potentially leading to functional cures; the suitability of ARCUS nucleases for gene elimination, insertion and excision and differentiation from other gene editing approaches due to its small size, simplicity and distinctive cut; the expected timing of regulatory processes (including filings such as IND's and CTA's and studies for PBGENE-HBV); expectations about operational initiatives, strategies, and further development of our programs; expectations about achievement of key milestones; and anticipated timing of clinical data. In some cases, you can identify forward-looking statements by terms such as "aim," "anticipate," "approach," "believe," "contemplate," "could," "designed," "estimate," "expect," "goal," "intend," "look," "may," "mission," "plan," "possible," "potential," "predict," "project," "pursue," "should," "strive," "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 to advance our programs; risks associated with raising additional capital and requirements under our current debt instruments and effects of restrictions thereunder; 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; our dependence on our ARCUS technology; the risk that other genome-editing technologies may provide significant advantages over our ARCUS technology; the initiation, cost, timing, progress, achievement of milestones and results of research and development activities, preclinical studies and clinical trials; 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; 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' or other licensees' ability to advance product candidates into, and successfully design, implement and complete, clinical or field trials; our or our collaborators' other licensees' 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; 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; the rate and degree of market acceptance of any of our product candidates; 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; our ability to effectively manage the growth of our operations; our ability to attract, retain, and motivate key executives and personnel; market and economic conditions; effects of system failures and security breaches; 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; insurance expenses and exposure to uninsured liabilities; effects of tax rules; risks related to ownership of our common stock; 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 Quarterly Report on Form 10-Q for the quarterly period ended March 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 presentation 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. Precision consults with various presentation speakers and compensates them for their time and expertise.

View source version on businesswire.com: https://www.businesswire.com/news/home/20240605698770/en/

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

Source: Precision BioSciences, Inc.