Precision BioSciences Submits First Clinical Trial Applications to Initiate Phase 1 Trial for PBGENE-HBV for the Treatment of Chronic Hepatitis B

September 30, 2024 at 7:01 AM EDT

- PBGENE-HBV is the only drug modality designed to target the root cause of disease by eliminating cccDNA and inactivating integrated HBV

- Clinical trial applications submitted for the first gene editing approach for chronic hepatitis B

- Final clinical candidate safety data and plans for the Phase 1 trial to be shared in November prior to the American Association for the Study of Liver Diseases meeting

DURHAM, N.C.--(BUSINESS WIRE)--Sep. 30, 2024-- Precision BioSciences, Inc. (Nasdaq: DTIL), an advanced gene editing company utilizing its novel proprietary ARCUS® platform to develop *in vivo* gene editing therapies for sophisticated gene edits, today announced that the Company submitted Clinical Trial Applications (CTA) to initiate a Phase 1 study evaluating PBGENE-HBV. PBGENE-HBV is the Company's wholly owned *in vivo* gene editing program designed to potentially cure chronic hepatitis B virus (HBV) by eliminating cccDNA, the key source of replicating hepatitis B virus, while also inactivating integrated HBV DNA in hepatocytes.

"The CTA submissions for PBGENE-HBV are important milestones for Precision as we pioneer this potentially curative therapy for chronic hepatitis B. These regulatory submissions are the culmination of our team's dedication, commitment and highly productive interactions with global regulators as we develop the first clinical stage *in vivo* gene editing program for chronic hepatitis B virus, recognized as one of the largest global public health problems by the World Health Organization with an estimated 300 million people afflicted globally," said Michael Amoroso, Chief Executive Officer of Precision BioSciences. "Our comprehensive regulatory package, supported by robust non-human primate (NHP) safety studies, the gold standard model for predicting safety in humans, and efficacy in numerous preclinical models of hepatitis B including in NHPs enables us to proceed with planned regulatory submissions in multiple markets around the world. We recently bolstered our clinical team expertise while building a world class hepatitis scientific advisory board to assist in guiding execution of our global Phase 1 trial. Our goal is clear, to generate robust clinical data for the patients afflicted with hepatitis B who are counting on us to significantly increase their chance of achieving a functional cure."

Dr. Murray Abramson, Senior Vice President, Head of Clinical Development added: "Current standard of care treatment with nucleos(t)ide analogs only offers 1-3% of patients a chance to achieve a functional cure. With our recent regulatory submissions, we are on the cusp of initiating a global first-in-human study for our wholly owned PBGENE-HBV program. PBGENE-HBV is specifically designed to provide a better chance for a functional cure for chronic hepatitis B by eliminating the root source of viral replication, known as cccDNA. This is the first approach to directly target and eliminate cccDNA. We are excited at the prospect of bringing this potentially curative therapy to patients living with hepatitis B. We look forward to initiating the Phase 1 study soon and expect to report data in 2025. More information about the safety of our clinical candidate and Phase 1 clinical trial will be communicated in November when we plan to share a robust overview of the program and our execution plans."

Precision is on track to submit additional regulatory applications as part of its global Phase 1 regulatory strategy for PBGENE-HBV. The next update on the PBGENE-HBV program is expected to take place before the American Association for the Study of Liver Diseases (AASLD) Annual Meeting in November. Details about how to participate in the update will be provided in advance.

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 is 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 (including PBGENE-HBV) 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 or providing a better chance of 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 and the acceptance of these filings by regulatory agencies); the translation of preclinical safety and efficacy studies and models to safety and efficacy in humans, the suitability of PBGENE-HBV for the treatment of hepatitis and the targeting of the root cause of the disease, 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, 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 Quarterly Report on Form 10-Q for the quarterly period ended June 30, 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/20240930788909/en/

Investor Contact:
Naresh Tanna
Vice President, Investor Relations
Naresh.Tanna@precisionbiosciences.com

Source: Precision BioSciences, Inc.