Precision BioSciences Hosts Virtual R&D Day Highlighting its Proprietary ARCUS Technology and Recent In Vivo Gene Editing Program Developments

September 12, 2023 at 7:00 AM EDT

- Company highlights new preclinical data for wholly-owned and partnered programs following prioritization of in vivo gene editing portfolio

- Expands portfolio with PBGENE-PMM as a potentially curative treatment for primary mitochondrial myopathy

- Virtual R&D Day featuring key opinion leaders and members of Precision's leadership and scientific teams to be held today at 9:00 AM ET

DURHAM, N.C.--(BUSINESS WIRE)--Sep. 12, 2023-- 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 plans to highlight its current development programs, pipeline updates, and underlying ARCUS platform technology during a virtual R&D Day to be held today at 9:00 AM ET.

"At Precision, we are moving forward with a renewed identity and focus on our core foundational strength as a gene editing company. During today's R&D Day, we are excited to highlight the potential of our ARCUS genome editing platform to develop differentiated programs enabled by the nature of ARCUS' unique cut, size, and simplicity," said Michael Amoroso, Chief Executive Officer at Precision BioSciences. "In addition to illustrating where we see a clear advantage for ARCUS, we will drill down on the novel attributes of ARCUS that inform our development program strategy. We look forward to highlighting the progress we are making across our robust wholly-owned and partnered *in vivo* gene editing portfolio."

"As we continue advancing our lead program PBGENE-HBV targeting hepatitis B virus (HBV) toward a 2024 clinical trial application (CTA) and/or investigational new drug (IND) application, we are pleased to introduce PBGENE-PMM, our first development candidate targeting mitochondrial DNA. PBGENE-PMM holds the potential to deliver a one-time, transformative treatment for patients with primary mitochondrial myopathy (PMM)," said Jeff Smith, Co-Founder and Chief Research Officer. "Together with PBGENE-HBV, we believe that both of these programs are emblematic of the unique advantages of our ARCUS technology. As we look ahead, we plan to drive organic development of programs focusing on more sophisticated edits such as viral or mutant mitochondrial genome elimination, defective gene excision, and gene insertion with ARCUS, while also establishing partnerships to rapidly advance programs."

Guest speakers will include:

- **Mark Sulkowski, M.D. -** *Chief, Division of Infectious Disease, John Hopkins University School of Medicine*
- **Carlos T. Moraes, Ph.D. -** *Lichtenstein Professor for Neurology, University of Miami Miller School of Medicine*
- **Michio Hirano, M.D. -** *Chief, Division of Neuromuscular Disorders, New York Presbyterian/Columbia University Irving Medical Center*

Highlighted R&D Day Topics

ARCUS Platform Overview

Precision will highlight key capabilities and differentiating characteristics of its proprietary ARCUS genome editing platform, which may enable ARCUS nucleases to drive more intended, defined outcomes.

Pipeline Overview and Updates to Development Programs

- **PBGENE-HBV** is being developed for the treatment of patients with chronic hepatitis B, with submission of a CTA and/or IND application targeted for 2024. The Company will show data highlighting the potential of PBGENE-HBV to eliminate covalently closed circular DNA and inactivate integrated HBV DNA to drive durable antigen loss, leading to a potential functional cure. In addition, Dr. Alan List, Chief Medical officer at Precision, will be joined by Dr. Mark Sulkowski for a fireside chat on the HBV landscape.
- Precision will announce **PBGENE-PMM** as a potentially first-in-class opportunity for treatment of m.3243 associated primary mitochondrial myopathy. Mitochondrial diseases are the most common hereditary metabolic disorder, affecting 1 in 4,300 people. PMM currently lacks a curative treatment and impacts approximately 50% of patients with mitochondrial disease. The high specificity and single component nature of Precision's mitoARCUS nucleases are designed to enable specific editing of mutant mitochondrial DNA while allowing the normal (wild-type) mitochondrial DNA to repopulate in the mitochondria and restore normal function. Precision expects to submit a CTA and/or IND in 2025. The Company will host a fireside chat between Dr. List, Dr. Carlos T. Moraes and Dr. Michio Hirano to discuss the mitochondrial disease space.
- **PBGENE-NVS** is being developed in collaboration with Novartis for patients with hemoglobinopathies, such as sickle cell disease and beta thalassemia. The companies intend to insert a therapeutic transgene *in vivo* as a potential one-time

transformative treatment that could overcome many of the hurdles present today with other therapeutic technologies, including those that are utilizing an *ex vivo* gene editing approach.

- **PBGENE-DMD** is being developed with Prevail Therapeutics, a wholly-owned subsidiary of Lilly, and is designed to utilize a pair of ARCUS nucleases, delivered by a single AAV, to excise an approximately 500,000 base pair mutation “hot spot” region from the dystrophin gene, resulting in a variant of the dystrophin protein that is functionally competent. The Company will highlight preclinical data demonstrating the potential of ARCUS *in vivo* gene editing for large gene excisions and that the edited dystrophin variant was observed in multiple tissue types frequently involved in progression of DMD, including skeletal muscle, heart, and diaphragm, enabling significantly improved muscle function.
- **iECURE-OTC** is being developed by iECURE as a potential treatment for neonatal onset ornithine transcarbamylase (OTC) deficiency with a target CTA and/or IND submission in 2023. The Company will highlight data demonstrating that iECURE-OTC showed stable gene insertion at one year and that efficient targeted insertion was achieved in non-human primates (NHPs) up to three months of age. In addition, 12-month follow-up biopsies continued to demonstrate durability, with gene insertion efficiency exceeding 20%, well above the expected threshold for clinical benefit.

Webcast Information

Registration for this virtual event and access to the live webcast is available at http://precision2023rdday.q4ir.com or under Events & Presentations in the Investors section of the Precision BioSciences website at https://investor.precisionbiosciences.com. The accompanying presentation will also be available on the Events & Presentations in the Investors section of the Precision BioSciences website at https://investor.precisionbiosciences.com. The dial-in conference call number is 1 (888) 800-8518. The conference ID number for the call is 2795912. An archived replay of the webcast will be available on the Company website for one year following the presentation.

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. ARCUS is a highly precise and versatile genome editing platform that was designed with therapeutic safety, delivery, and control in mind. 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.

Forward-Looking Statements

This press release contains forward-looking statements within the meaning of the Private Securities Litigation Reform Act of 1995. The Company intends such forward-looking statements to be covered by the safe harbor provisions for forward-looking statements contained in Section 27A of the Securities Act of 1933, as amended, and Section 21E of the Securities Exchange Act of 1934, as amended. 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 ARCUS’s potential editing efficiency and differentiating aspects and the ability for the ARCUS genome editing platform to develop differentiated programs; the suitability of ARCUS nucleases for gene or viral elimination, large excision, gene insertion, and other complex gene editing approaches to drive defined outcomes; the expected timing of regulatory processes; expectations about our operational initiatives and business strategy; expectations around the Company’s partnerships; and expectations about achievement of key milestones. The words “aim,” “anticipate,” “approach,” “believe,” “contemplate,” “could,” “designed”, “estimate,” “expect,” “goal,” “intend,” “look,” “may,” “mission,” “plan,” “possible,” “potential,” “predict,” “project,” “promise,” “pursue,” “should,” “target,” “will,” “would,” and other similar words or expressions, or the negative of these words or similar words or expressions, are intended to identify forward-looking statements, though not all forward-looking statements use these words or 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; 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; 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 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 June 30, 2023, as any such factors may be updated from time to time in our other filings with the Securities and Exchange Commission (“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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