Precision BioSciences Announces Presentation at UMDF Mitochondrial Medicine 2024 Supporting Advancement of PBGENE-PMM Program Towards IND and/or CTA in 2025

June 27, 2024 at 7:01 AM EDT

DURHAM, N.C.--(BUSINESS WIRE)--Jun. 27, 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, including gene elimination, insertion, and excision, is presenting data today at the United Mitochondrial Disease Foundation's (UMDF) Mitochondrial Medicine 2024 Conference being held in Cleveland, Ohio from June 26-29, 2024.

"Our team is proud to share details from our lead PBGENE-PMM program targeting m.3243 mitochondrial disease and showcase the broader applicability of ARCUS to target other mitochondrial mutations," said Jeff Smith, Co-Founder and Chief Research Officer at Precision Biosciences. "Our work targeting mitochondrial DNA takes advantage of the simplicity of ARCUS as a single component editor with both recognition and catalytic activity all in one protein that does not require a guide-RNA like most other gene editing tools. PBGENE-PMM is designed to eliminate mutant mitochondrial DNA leaving normal functioning wild-type mitochondrial DNA intact to repopulate the cell, resulting in a shift in heteroplasmy and improvement in mitochondrial function. Given ARCUS' ability to target the root cause of disease, we are excited by our data generated to date and are working diligently to advance PBGENE-PMM towards an Investigational New drug (IND) and/or Clinical Trial Application (CTA) in 2025."

Presentation Details:
Title: Shifting Heteroplasmy with PBGENE-PMM: Gene Editing Therapy for m.3243A>G Associated Mitochondrial Myopathy
Presenter: Wendy Shoop, PhD, Senior Scientist, Precision Biosciences
Date and Time: Thursday, June 27, 2024, 11:00 AM EDT.

The data for PBGENE-PMM, Precision's lead mitochondrial editing therapeutic program, demonstrated ARCUS' ability to localize exclusively to mitochondria, avoiding any detectable off-target editing in the nuclear genome, and generate substantial shifts in heteroplasmy and improvements in mitochondrial function.

The data showcases the ability of ARCUS to selectively eliminate mutant mitochondrial DNA, including the common deletion (del_mtDNA4977), mouse m.5024T, and human m.3243G, which highlights the broad applicability of the ARCUS platform for heteroplasmic mitochondrial DNA mutations.

About PBGENE-PMM

PBGENE-PMM is our wholly owned, first of its kind treatment for m.3243 mitochondrial disease. Mitochondrial diseases are the most common hereditary metabolic disorder, affecting 1 in 4,300 people. This disease lacks a curative treatment and impacts approximately 30-40% of patients with mitochondrial disease. In the Company's 2023 publication in *Nature Metabolism*, Precision presented data highlighting the high specificity and single component nature of the PBGENE-PMM and ability to specifically edit and eliminate mutant mitochondrial DNA while allowing wild-type (normal) mitochondrial DNA to repopulate in the mitochondria, thus restoring normal function. Precision expects to submit a CTA and/or IND for this program in 2025.

About ARCUS

ARCUS is a proprietary genome editing technology discovered and developed by scientists at Precision BioSciences. It uses sequence-specific DNA-cutting enzymes, or nucleases, that are designed to either insert (knock-in), excise (knock-out), eliminate, or repair DNA of living cells and organisms. ARCUS is based on a naturally occurring genome editing enzyme, I-CreI, that evolved in the algae *Chlamydomonas reinhardtii* to make highly specific cuts in cellular DNA and stimulate gene insertion at the cut site by homologous recombination. Precision's platform and products are protected by a comprehensive portfolio including more than 130 patents to date.

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 therapeutic potential of an ARCUS gene editing approach for the treatment of m.3243-associated mitochondrial disease including the common deletion (del_mtDNA4977) and mouse m.5024T, the ability of ARCUS to enter the mitochondrial matrix and preferentially target and eliminate mutant m.3243G mtDNA with high specificity and without off-target activity, anticipated timing of a CTA and/or IND filing, the ability of mitoARCUS to shift heteroplasmy, and expected safety, efficacy, and benefit of our gene editing approaches. 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 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 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.

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