Precision BioSciences Receives U.S. Patent Allowance Covering PBGENE-PMM for m.3243-Associated Mitochondrial Diseases

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DURHAM, N.C.--(BUSINESS WIRE)--Sep. 27, 2023-- 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 insertion, excision, and elimination, today announced it has received a Notice of Allowance from the U.S. Patent and Trademark Office (USPTO) for U.S. Patent Application No. 18/161,560, titled “Engineered Meganucleases That Target Human Mitochondrial Genomes.” Once issued, the patent arising from this application will have a standard expiration date in April 2042.

The allowed composition of matter claims in this U.S. application encompass a mitochondria-targeted ARCUS nuclease (mitoARCUS) that is designed to specifically target, cleave, and eliminate mutant mitochondrial DNA comprising an m.3243A>G mutation. The m.3243A>G mutation is one of the most common pathogenic mitochondrial DNA mutations, differing from wild-type (normal) mitochondrial DNA by a single base change, and is associated with the development of a number of disorders, including primary mitochondrial myopathies that primarily affect skeletal muscle, and mitochondrial encephalomyopathy, lactic acidosis and stroke-like episodes (MELAS).

Precision recently announced PBGENE-PMM, the Company’s clinical candidate targeting mutant mitochondrial DNA, as a potentially first-in-class opportunity for treatment of m.3243 associated primary mitochondrial myopathy. Utilizing the claimed mitoARCUS nuclease, PBGENE-PMM is designed to target and eliminate mutant mitochondrial DNA, allowing for repopulation by wild-type mitochondrial DNA and restoration of mitochondrial function.

“The high specificity and single component nature of Precision’s mitoARCUS nucleases are designed to enable specific elimination of mutant mitochondrial DNA while allowing the normal mitochondrial DNA to repopulate in the mitochondria and reestablish normal function,” said Jeff Smith, PhD, Co-Founder and Chief Research Officer at Precision BioSciences. “PBGENE-PMM holds the potential to deliver a one-time, transformative treatment for patients with primary mitochondrial myopathy.”

Unlike CRISPR-based gene editing tools, mitoARCUS nucleases are able to gain access to mitochondria because they are small, single-component proteins that integrate DNA-binding and DNA-cleavage and do not require a nucleic acid, such as a guide RNA, for targeting.

“The fact that mitoARCUS can be delivered directly to mitochondria, and has the specificity to distinguish a single base pair difference in the m.3243 A>G mutation, makes PBGENE-PMM a very important potential treatment candidate for patients suffering from m.3243 associated primary mitochondrial myopathy,” said Carlos Moraes, PhD, Esther Lichtenstein Professor of Neurology, and Cell Biology and Anatomy at the University of Miami Miller School of Medicine, and co-inventor of the allowed application.

This U.S. application is jointly owned by Precision and the University of Miami, which has exclusively licensed the rights to the application to the Company.

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.

About Mitochondria and Primary Mitochondrial Myopathy

Mitochondria comprise multiple copies of a circular DNA referred to as mitochondrial DNA, which encodes for 13 subunits of the oxidative phosphorylation (OXPHOS) system, 2 rRNAs, and 22 tRNAs that are all necessary to support mitochondrial function. It is believed that a shift in mitochondrial DNA heteroplasmy toward wild-type (normal) may provide therapeutic benefit for patients, and not all mutant mitochondrial DNA must be eliminated to achieve improvements in symptoms. Rather, mutant mitochondrial DNA levels only need to be shifted below a disease threshold level.

Mitochondrial diseases that arise from mutations in mitochondrial DNA are the most common hereditary metabolic disorder, affecting 1 in 4,300 people. Primary mitochondrial myopathy is characterized by severe fatigue and can affect skeletal muscle, and other high energy organs such as the brain, eyes, ears and heart. Primary mitochondrial myopathy currently lacks curative treatment and impacts approximately 50% of patients with mitochondrial disease.

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 and Section 21E of the Exchange Act. 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 insertion, excision, and elimination, including the elimination of mutant mitochondrial DNA, the clinical development, nomination, and goals of our PBGENE-PMM program, the potential for a shift in heteroplasmy toward wild-type mitochondrial DNA to restore mitochondrial function, and therapeutic potential of an ARCUS gene editing approach for the treatment of m.3243-associated mitochondrial diseases. The words “aim,” “anticipate,” “approach,” “believe,” “contemplate,” “could,” “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 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; 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; pending and potential 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 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; our or our collaborators’ 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’ 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 COVID-19 pandemic and variants thereof, or any pandemic, epidemic or outbreak of an infectious disease; 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 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 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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