Precision BioSciences Receives FDA Rare Pediatric Disease Designation for PBGENE-DMD for the Treatment of Duchenne Muscular Dystrophy

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DURHAM, N.C.--(BUSINESS WIRE)--Jun. 25, 2025-- Precision BioSciences, Inc. (Nasdaq: DTIL), a clinical stage gene editing company utilizing its novel proprietary ARCUS® platform to develop *in vivo* gene editing therapies for diseases with high unmet need, today announced that the U.S. Food and Drug Administration (FDA) has granted Rare Pediatric Disease Designation for PBGENE-DMD for the treatment of Duchenne muscular dystrophy (DMD).

“The receipt of Rare Pediatric Disease Designation highlights the significant unmet need that necessitates new therapeutic options for boys living with DMD,” said Cindy Atwell, Chief Development and Business Officer at Precision BioSciences. “Our first-in-class PBGENE-DMD program, which leverages the differentiated capabilities of our ARCUS gene editing platform to excise exons 45-55 of the dystrophin gene, holds the potential to drive meaningful improvement and durable functional benefit over time for up to 60% of patients with DMD. Our approach is designed to permanently edit a patient’s own DNA sequence, resulting in naturally-produced, near full-length dystrophin protein known to be functional in humans. We look forward to completing the final IND-enabling toxicology studies currently underway and generating initial clinical data which is expected in 2026.”

The FDA grants Rare Pediatric Disease Designation to therapies targeting serious and life-threatening diseases that affect fewer than 200,000 people in the U.S. and primarily affect individuals under the age of 18. Approximately 15,000 people in the U.S. alone are living with Duchenne muscular dystrophy.

With this designation, Precision may be eligible to receive a Priority Review Voucher upon FDA approval of PBGENE-DMD. The Priority Review Voucher program is designed to incentivize drug development for serious rare pediatric diseases. If awarded, a Priority Review Voucher can be redeemed by the original sponsor to receive FDA priority review for a different product. In addition, the Priority Review Voucher can also be sold to a different sponsor, serving as a source of non-dilutive capital to the original sponsor.

About PBGENE-DMD

PBGENE-DMD is Precision’s development program for the treatment of DMD. The approach uses two complementary ARCUS nucleases delivered via a one-time administration in a single AAV to excise exons 45-55 of the dystrophin gene with the aim of restoring near-full length dystrophin protein within the body to improve functional outcomes. PBGENE-DMD is intended to address more than 60% of the DMD patient population.

In preclinical studies, PBGENE-DMD demonstrated the ability to target key muscle types involved in the progression of DMD and produced significant, durable functional improvements in a humanized DMD mouse model. PBGENE-DMD restored the body’s ability to produce a near full length functional dystrophin protein across multiple muscles, including cardiac tissue and various key skeletal muscle groups. In addition, PBGENE-DMD edited satellite muscle stem cells, believed to be critical for long-term durability and sustained functional improvement.

About Precision BioSciences, Inc.

Precision BioSciences, Inc. is a clinical stage 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 such as in the DMD program).

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 and our partners’ and licensees’ product candidates and gene editing approaches; the potential of PBGENE-DMD to drive meaningful improvement in functional and durable benefit over time for up to 60% of patients with DMD; the design on PBGENE-DMD to permanently edit a patient’s own DNA sequence, resulting in naturally-produced, near full-length dystrophin protein proven known to be functional in humans; the potential value of a Priority Review Voucher(if awarded) including priority review for a different product or sale to another sponsor; the recent sale of a Priority Review Voucher for more than $150 million; the approach of using a single AAV to deliver PBGENE-DMD to excise exons 45-55 of the dystrophin gene with the aim of restoring a near-full length dystrophin protein within the body to improve functional outcomes; the expected timing of regulatory processes and clinical operations (including IND and/or CTA filings, studies, enrollment and clinical data for PBGENE-DMD; and anticipated timing of clinical data. In some cases, you can identify forward-looking statements by terms such as “aim,” “anticipate,” “appear,” “approach,” “believe,” , “confidence”, “contemplate,” “could,” “design” “designed,” “estimate,” “expect,” “goal,” “intend,” “look,” “may,” “mission,” “plan,” “possible,” “potential,” “predict,” “project,” “pursue,” “should,” “strive,” “suggest,” “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 March 31, 2025, 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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