Precision BioSciences Highlights New Preclinical Data for PBGENE-DMD Further Supporting Advancement of Novel Gene Editing Approach for the Treatment of Duchenne Muscular Dystrophy Towards Clinic

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- New preclinical data for the PBGENE-DMD final clinical candidate demonstrates an increase in dystrophin positive muscle cells across key muscle types, potentially driven by editing of muscle satellite cells -

- PBGENE-DMD is a first-in-class in vivo gene editing approach for up to 60% of Duchenne Muscular Dystrophy patients, specifically those impacted by dystrophin mutations in the 'hot spot' region between exons 45-55 -

- Precision targeting to submit an Investigational New Drug (IND) and/or Clinical Trial Application (CTA) for PBGENE-DMD in 2025 with clinical data expected in 2026 -

DURHAM, N.C.--(BUSINESS WIRE)--Jul. 16, 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 updated additional preclinical data further validating the scientific rationale supporting the rapid development of PBGENE-DMD, the Company's first-in-class *in vivo* gene editing approach for Duchenne muscular dystrophy (DMD), towards first-in-human clinical investigation.

"In a long-term durability study in a DMD diseased mouse model, we have observed up to a three-fold increase in dystrophin-positive muscle cells between three and nine months in the quadricep, gastrocnemius (calf), heart, and diaphragm. In the gastrocnemius, up to 85% of cells were dystrophin-positive," said Cassie Gorsuch, PhD., Chief Scientific Officer at Precision BioSciences. "These new data build upon the preclinical data shared at ASGCT in May 2025, demonstrating that PBGENE-DMD treatment resulted in significant and sustained improvement of maximum force output at the same three- and nine-month timepoints. This broad increase in dystrophin-positive cells, along with the increased dystrophin protein detected in tissues, further validates the improved muscle function that was observed over time and may be attributable to edited satellite cells, which were also observed in this study. We believe these results demonstrate the unique potential of the PBGENE-DMD gene editing approach to produce a sustained functional benefit without the need for AAV persistence, which is required of microdystrophin approaches. We look forward to presenting the complete dataset at a future scientific conference. Given the totality of preclinical evidence, we remain excited and steadfast in advancing this program towards clinic."

Currently, there are no approved treatments or treatments in development that significantly improve muscle function over time to beneficially alter the long-term prognosis of DMD. PBGENE-DMD is the first *in vivo* gene editing program that has the potential to transform the treatment paradigm and deliver durable functional improvement for most patients, as up to 60% of those afflicted carry mutations in the 'hot spot' region between exons 45-55. Precision's 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. The final IND-enabling toxicology studies are currently underway with IND and/or CTA filing targeted in 2025 and initial clinical data expected in 2026. The Company believes that its current cash runway will be sufficient to progress both PBGENE-HBV, its current Phase 1 asset, and PBGENE-DMD through Phase 1 clinical readouts.

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 up to 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's two lead programs, PBGENE-HBV, for chronic Hepatitis B, and PBGENE-DMD, for Duchenne Muscular Dystrophy, are focused on areas with large patient populations with high unmet need. 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 enable ARCUS nucleases to drive more intended, defined therapeutic outcomes. Using ARCUS, the Company's pipeline prioritizes *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. 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 including PBGENE-DMD; 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 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 increased dystrophin protein detected in the tissues and improved muscle function that was observed over time in the DMD mouse model may be attributable to edited satellite cells could be driving sustained replenishment of edited myocytes, resulting in increased dystrophin protein expression, dystrophin -positive cells, and improved muscle function over time; 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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Investor and Media Contact:
Naresh Tanna
Vice President of Investor Relations
naresh.tanna@precisionbiosciences.com

Source: Precision BioSciences, Inc.