# Precision BioSciences Accelerates Development of PBGENE-DMD Within its Wholly Owned Organic Pipeline and Highlights Pre-clinical Evidence at the American Society of Gene and Cell Therapy (ASGCT) Annual Meeting

May 14, 2025 at 4:30 PM EDT

*- PBGENE-DMD is a first-in-class in vivo gene editing approach for the majority of Duchenne Muscular Dystrophy patients impacted by dystrophin mutations in the most common 'hot spot' region between exons 45-55 -*

*- Final clinical candidate PBGENE-DMD demonstrates compelling preclinical data for durably improving functional benefit over time -*

*- 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 -*

*- Precision to host a webcast and conference call on Thursday, May 15, 2025 at 8:00 AM ET -*

DURHAM, N.C.--(BUSINESS WIRE)--May 14, 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 the strategic prioritization and acceleration of PBGENE-DMD, the Company's first-in-class *in vivo* gene editing approach for Duchenne Muscular Dystrophy (DMD) and highlighted a PBGENE-DMD poster presentation at the American Society of Gene and Cell Therapy (ASGCT) Annual Meeting being held May 13-17, 2025, in New Orleans, Louisiana.

"Nomination and acceleration of PBGENE-DMD as our second wholly owned program is a result of the compelling preclinical evidence we have generated to date," said Michael Amoroso, President and Chief Executive Officer of Precision BioSciences. "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 this devastating disease. 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. Based on these data, the significant unmet need in DMD and the clear regulatory guidance established for new therapeutics in DMD, we are committed to advancing PBGENE-DMD to the next stage of development. We look forward to rapidly advancing this program toward the clinic as our second wholly owned program after PBGENE-HBV and further establishing the therapeutic potential of ARCUS *in vivo* gene editing."

DMD is a genetic disease caused by mutations in the dystrophin gene that prevent production of the dystrophin protein and affects approximately 15,000 patients in the U.S. alone. There are currently no approved therapies that can drive significant and durable functional muscle improvements. PBGENE-DMD employs two complementary ARCUS nucleases delivered in a single AAV to excise exons 45-55 of the dystrophin gene with the aim of restoring the body's natural production of a functional dystrophin protein.

"PBGENE-DMD has the potential to provide a one-time, durable intervention that could allow for lifelong benefits in muscle regeneration and function. Preclinical models have shown that PBGENE-DMD results in significant and sustained improvement of maximum force output by restoring the human body's production of a functional, near full-length dystrophin protein," added Cassie Gorsuch, Ph.D., Chief Scientific Officer at Precision BioSciences. "Approximately one in every 3,500–5,000 males in the United States is afflicted with DMD, and these patients have limited treatment options. Our prioritization of PBGENE-DMD in conjunction with long term, preclinical functional improvement reinforces our belief in the program and its potential to address significant unmet need for patients living with DMD."

In preclinical data being presented at ASGCT, PBGENE-DMD demonstrated significant and durable functional improvement in a humanized DMD mouse model. Following AAV delivery, PBGENE-DMD restored the body's ability to produce a functional dystrophin protein broadly across multiple muscles, including cardiac and skeletal muscles. Over the course of 9 months, mice treated with PBGENE-DMD showed increased dystrophin protein expression resulting in substantial and sustained functional muscle improvement. In addition, PBGENE-DMD-edited dystrophin mRNA transcript in muscle satellite stem cells, which are progenitor cells for new muscle cells, supports the potential for long-term durability.

"Precision's ARCUS gene excision approach targeting exons 45-55 represents a novel approach to addressing the underlying disease for the majority of patients with Duchenne muscular dystrophy," said Debra Miller, founder and CEO of CureDuchenne. "We are excited by the potential of this approach and look forward to Precision educating the DMD community about it at our upcoming FUTURES National Conference on May 24." CureDuchenne is a nonprofit organization recognized as the global leader in research, patient care and innovation for improving and extending the lives of those with Duchenne muscular dystrophy.

Precision is working diligently and targeting to file an IND and/or CTA in 2025 with clinical data anticipated in 2026. The Company believes that its current cash runway will be sufficient to progress both PBGENE-HBV, its current Phase 1 clinical program, and PBGENE-DMD through Phase 1 clinical readouts.

In order to accelerate development of PBGENE-DMD and maintain operational capability to pursue PBGENE-HBV and PBGENE-DMD through Phase 1 clinical results, Precision plans to pause development of PBGENE-3243, its potential treatment for m.3243-associated mitochondrial disease, and will stage future development alone or with partners following completion of the Phase 1 ELIMINATE-B trial and after the PBGENE-DMD program enters the clinic. Precision has completed pre-IND discussions with regulators for PBGENE-3243 and the final clinical candidate is ready to commence

toxicology studies. “We remain excited about the potential for PBGENE-3243 to help people living with m.3243 mitochondrial diseases and remain committed to those afflicted with m.3243 associated mitochondrial disease in the future, alone or through partnerships,” added Mr. Amoroso.

**Conference Call Information**

Precision BioSciences will host a conference call on Thursday, May 15 at 8:00 am ET. To access the live conference call, participants may register here. The live audio webcast of the call will be available in the Investors section under Events & Presentations at investor.precisionbiosciences.com. An archived replay of the webcast will be available for approximately 30 days following the event.

**PBGENE-DMD Presentation Details:**

**Title:** ARCUS-Mediated Gene Editing Excision of Exons 45-55 of the Human Dystrophin Gene using PBGENE-DMD Leads to Functional Dystrophin Protein and Durable Restoration of Skeletal Muscle-Function In Vivo for the Treatment of Duchenne Muscular Dystrophy

**Session:** Poster Reception

**Date and Time:** Wednesday, May 14, 2025, 5:30 PM - 7:00 PM CT

**Location:** Poster Hall I2

**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. 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. 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 editing efficiency, and the suitability of ARCUS nucleases for gene insertion, gene elimination and gene excision and differentiation from other gene editing approaches; the ability, aim, or therapeutic potential of PBGENE-DMD to transform the treatment paradigm and deliver durable functional improvement, restore the body’s natural production of a functional dystrophin protein that more closely resembles normal dystrophin, provide a one-time, durable intervention that could allow for lifelong benefits in muscle retention and function, or for significant functional dystrophin protein production across cardiac and skeletal muscles at levels expected to provide therapeutic benefit; demonstrating breadth and therapeutic potential of ARCUS in vivo gene editing pipeline to drive near-term and long-term value and commercial opportunity; the expected timing of regulatory processes and clinical operations (including IND and/or CTA filings, studies, enrollment and clinical data for PBGENE-DMD and PBGENE-HBV); the ability of PBGENE-DMD to edit muscle satellite stem cells suggesting a potential for long-term durability; the translation of results in preclinical studies of ARCUS nucleases to clinical studies in humans; expectations about our and our partners’ operational initiatives, strategies, and further development of our programs; the sufficiency of our cash runway to progress both PBGENE-HBV and PBGENE-DMD through Phase 1 clinical readouts; expectations about achievement of key milestones and receipt of any milestone, royalty, or other payments; expectations regarding our liquidity and capital resources; expectations to continue development and timing of PBGENE-3243; and anticipated timing of clinical data. In some cases, you can identify forward-looking statements by terms such as “aim,” “anticipate,” “appear,” “approach,” “believe,” “contemplate,” “could,” “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 Annual Report on Form 10-K for the year ended December 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 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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