Precision BioSciences Announces Late-Breaking Poster Presentation at the 30th Annual International Congress of the World Muscle Society

September 30, 2025 at 7:01 AM EDT

- Data to highlight latest long-term preclinical efficacy and durability data supporting PBGENE-DMD for the treatment of Duchenne Muscular Dystrophy -

- On track to file an IND and/or CTA by the end of 2025; clinical data anticipated in 2026-

DURHAM, N.C.--(BUSINESS WIRE)--Sep. 30, 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 high unmet need diseases, today announced a late-breaking poster presentation at the 30th Annual International Congress of the World Muscle Society (WMS) being held October 7-12, 2025, in Vienna, Austria.

"We look forward to sharing the latest preclinical data for PBGENE-DMD at the upcoming World Muscle Society congress," said Dr. Cassie Gorsuch, PhD, Chief Scientific Officer at Precision BioSciences. "There remains a significant need for new therapeutic options for patients living with DMD, and PBGENE-DMD holds the potential to be a first-in-class gene editing approach for up to 60% of patients who are impacted by dystrophin mutations between exons 45-55. The preclinical data to be presented demonstrates PBGENE-DMD's ability to drive significant and sustained improvements in muscle function over time through increased frequency of dystrophin positive cells, increased dystrophin protein expression, and satellite stem cell editing. These encouraging results underscore PBGENE-DMD's potential to transform the treatment paradigm for patients with DMD, and we remain on track to file an IND and/or CTA filing by the end of 2025 with clinical data anticipated in 2026."

Late-Breaking Poster Presentation Details:

Abstract Title: Treatment with PBGENE-DMD results in durable improvements in muscle function over time through increased dystrophin expression and dystrophin-positive cells
Session: Poster Session 4; Late-Breaking Poster Presentations
Presenter: Cassie Gorsuch, Ph.D., Chief Scientific Officer, Precision Biosciences
Date and Time: Friday, October 10, 2025, 3:45-4:45 PM CET

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. Utilizing two ARCUS nucleases delivered by a single AAV, PBGENE-DMD demonstrated durable improvements in muscle function over time through increased dystrophin expression and dystrophin-positive cells. In a DMD mouse model, PBGENE-DMD was administered at doses up to $1x10^{14}$ vg/kg, and mice were evaluated at 3- and 9-months post-dosing for molecular and functional outcomes. Following treatment, dystrophin protein was detected in all muscles evaluated, with increased expression observed at 9 months versus prior timepoints in the quadriceps, gastrocnemius, heart, and diaphragm resulting in substantial and sustained functional muscle improvement. Additionally, an increase in dystrophin-positive muscle cells was observed in all muscles, with up to 85% dystrophin-positive cells in the gastrocnemius. The maximum force output was significantly improved over untreated DMD mice at 3-, 6- and 9-months post-treatment, highlighting strong durability of PBGENE-DMD outcomes. ARCUS-edited dystrophin transcript was also observed in $PAX7^+$ cells, a marker for satellite stem cells, supporting the potential for long-term durability.

Precision continues to advance the final toxicology studies with an anticipated investigational new drug (IND) and/or clinical trial application (CTA) filing targeted by the end of 2025 with initial clinical data expected in 2026.

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 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 such as in the Company's PBGENE-HBV program), and excision (removing a large portion of a defective gene by delivering two ARCUS nucleases in a single AAV such as in the Company's 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 key advantages of ARCUS and its key capabilities and differentiating characteristics; the potential of PBGENE-DMD to be a first-in-class *in vivo* gene editing approach addressing up to 60% of DMD patients; expectations on accelerated development of the PBGENE-DMD program; the unique potential of PBGENE-DMD to achieve significant and sustained improvements in muscle function and an increase of dystrophin protein in muscle and satellite stem cell; PBGENE-DMD's potential to transform the treatment paradigm for DMD; we remain on track to file an IND and/or CTA filing by the end of 2025 with clinical data anticipated in 2026.the expected timing and opportunities of regulatory processes (including filing of an IND and/or CTA for PBGENE-DMD by the end of 2025 with clinical data anticipated in 2026). In some cases, you can identify forward-looking statements by terms such as "aim," "anticipate," "approach," "believe," "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 June 30, 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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