Precision BioSciences Presents New Preclinical Data Supporting the Advancement of PBGENE-DMD into Clinic at the American Society of Gene & Cell Therapy 2026 Annual Meeting

May 14, 2026 at 7:01 AM EDT

– New preclinical data show PBGENE-DMD drove up to a 3x higher dystrophin protein restoration in skeletal muscle and up to 12x higher in respiratory muscle in early-juvenile mice compared to late-juvenile mice –

– Findings further support evaluation of PBGENE-DMD in DMD patient populations as young as 2 years of age –

– Greater efficacy with earlier intervention further differentiates PBGENE-DMD from microdystrophin approaches –

DURHAM, N.C.--(BUSINESS WIRE)--May 14, 2026-- 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 new preclinical data from its PBGENE-DMD program in an oral presentation at the American Society of Gene & Cell Therapy (ASGCT) 2026 Annual Meeting in Boston, Massachusetts. The new data show that treatment with PBGENE-DMD in early-juvenile mice resulted in significantly higher efficacy across key skeletal and respiratory muscles than treatment in late-juvenile mice over a comparable timeframe. This new data further supports evaluating PBGENE-DMD in younger DMD patient populations, including the 2- to 3-year-old patients, who are a key demographic of the ongoing Phase 1/2 FUNCTION-DMD trial evaluating PBGENE-DMD in boys ages 2 to 7.

The greater efficacy observed in early-juvenile mice is also expected to be an important point of differentiation for PBGENE-DMD versus microdystrophin approaches. With microdystrophin approaches the AAV dilution effect driven by muscle growth and turnover would be expected to be even more pronounced in younger DMD patients.

"These new data give us a clear preclinical rationale for treating DMD as early as possible. By directly comparing early- and late-juvenile mice, we showed that intervening earlier translated into substantially greater dystrophin restoration in the skeletal and respiratory muscles that matter most for long-term outcomes. This is important because the FUNCTION-DMD trial is designed to treat children between the ages of two and seven years," said Cassie Gorsuch, Ph.D., Chief Scientific Officer of Precision BioSciences. "These results also reinforce a meaningful point of differentiation for PBGENE-DMD. Because we are correcting the gene rather than delivering a synthetic transgene, the durability of effect would not be expected to be subject to the AAV dilution that affects microdystrophin approaches as young patients grow, a limitation that becomes more pronounced the younger the patient."

New preclinical data: strong efficacy in early-juvenile mice (age 2 weeks, equivalent to a patient population aged 2–3 years)

- PBGENE-DMD in early-juvenile mice achieved up to 3x higher dystrophin protein restoration in skeletal muscle, and up to 12x higher dystrophin protein restoration in respiratory muscle, compared with late-juvenile mice at equivalent dose levels.
- Strong efficacy which exceeded the expected dystrophin protein restoration therapeutic threshold of 5% was observed in respiratory muscle tissues, with mice achieving up to 12% dystrophin restoration in the diaphragm and up to 30% in the intercostals, muscles whose function is critical to preventing respiratory failure in patients with DMD.
- PBGENE-DMD drove high levels of dystrophin-positive fibers in early-juvenile mice, with levels 2–3x higher in skeletal and respiratory muscle tissues than in late-juvenile mice after three months, reaching up to 70% dystrophin-positive fibers.
- Similar therapeutic efficacy was achieved in cardiac muscle in both early- and late-juvenile mice.

Building on a growing body of preclinical evidence

These new data extend Precision's previously reported preclinical findings supporting the safety, efficacy, and durability profile of PBGENE-DMD:

- A toxicology study in a humanized DMD mouse model showed that PBGENE-DMD treatment led to greater than 45% reduction in serum creatine kinase across multiple dose levels, alongside improvements in muscle pathology relative to vehicle-treated controls, supporting the safety profile of the program.
- Beyond safety, PBGENE-DMD has demonstrated sustained efficacy over time through dystrophin protein restoration and dystrophin-positive myofibers, translating into durable muscle function. Treated mice maintained up to 92% of the maximum force output of non-diseased animals, while untreated, diseased mice showed progressively declining force output.

Presentation details

Abstract title: PBGENE-DMD gene editing drives safe, efficacious, and durable functional improvement in a humanized Duchenne muscular dystrophy mouse model

Session: Emerging molecular therapeutic strategies for muscular dystrophies

Presenter: Adam Mischler, Ph.D., DMD Research Lead, Precision BioSciences

Date and time: Thursday, May 14, 2026, 8:45 A.M. ET

About PBGENE-DMD, A Muscle-Targeted Excision Program

PBGENE-DMD is Precision's development program for the treatment of Duchenne Muscular Dystrophy (DMD), a devastating genetic disease caused by mutations in the dystrophin gene that prevents production of the dystrophin protein, which is essential for maintaining muscle structural integrity and function. DMD affects approximately 15,000 patients in the U.S. alone, and there are currently no approved therapies capable of driving significant, durable functional improvements over time.

PBGENE-DMD is designed to durably improve function for approximately 60% of patients with DMD by employing two complementary ARCUS nucleases, delivered using a single AAV, to excise exons 45-55 of the dystrophin gene, restoring expression of a near full-length dystrophin protein. Compared with DMD, deletion of exons 45-55 is often associated with a more mild prognosis for patients. This protein more closely resembles normal dystrophin than synthetic, truncated microdystrophin approaches, which currently offer minimal proven functional benefit. Precision's Phase 1/2 FUNCTION-DMD study is expected to enroll ambulatory DMD patients with mutations between exons 45 and 55, which impact approximately 60% of boys with DMD. The clinical trial will employ an appropriate immune modulation regimen and safety monitoring program to treat patients at world class specialized DMD clinical sites.

PBGENE-DMD was granted Orphan Drug Designation by the FDA in July 2025. The PBGENE-DMD program is eligible for a Priority Review Voucher (PRV) via the Rare Pediatric Disease Priority Review Voucher (PRV) program, which was signed into law on February 3, 2026, as part of the Consolidated Appropriations Act of 2026. PBGENE-DMD received Fast Track designation from the FDA in February 2026.

Further details on the trial can be found on Precision's website and **clinicaltrials.gov** identifier NCT07429240.

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. These features are intended for ARCUS nucleases to drive more defined therapeutic outcomes. Using ARCUS, the Company's pipeline is comprised of clinical stage 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 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 PBGENE-DMD program) and gene insertion (inserting DNA into gene to cause expression/add function).

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; expectations about operational initiatives, strategies, further development, or timing of additional updates or data releases of PBGENE-DMD, timing and progress of IRB processes and site activations following IND clearance for the PBGENE-DMD program and FUNCTION-DMD trial; the design of PBGENE-DMD to improve function over time and address more than 60% of patients with DMD; the potential for PBGENE-DMD to provide durable functional improvement with a single dose of AAV; translation of results in preclinical studies of ARCUS nucleases to clinical studies in humans; that findings from a preclinical study support evaluation of PBGENE-DMD in younger DMD patient populations, including the 2- to 3-year-olds who are a key demographic of the Phase 1/2 FUNCTION-DMD trial, that greater efficacy with earlier intervention further differentiates PBGENE-DMD from microdystrophin approaches, where the AAV dilution effect from muscle growth would be expected to be more pronounced in younger patients; that intervening earlier in a preclinical model resulted in substantially greater dystrophin restoration in the skeletal and respiratory muscles that matter most for long-term outcomes; and expectations around the preclinical and clinical development and demonstrated, potential and expected safety, efficacy, durability, and benefit of PBGENE-DMD. In some cases, you can identify forward-looking statements by terms such as "aim," "anticipate," "approach," "belief", "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 March 31, 2026, 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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