Precision BioSciences Presents Preclinical PBGENE-DMD Data Highlighting Durable Dystrophin Expression and Functional Benefit at the Muscular Dystrophy Association Clinical & Scientific Conference 2026

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– Findings reinforce differentiated *in vivo* gene editing approach designed for durable functional muscle improvement through satellite cell editing –

– PBGENE-DMD treatment in a humanized DMD mouse model demonstrates improvements in muscle pathology and biomarkers of muscle damage –

– PBGENE-DMD shows durable dystrophin protein restoration in humanized DMD mice across key muscle groups, including cardiac, diaphragm, and skeletal muscles –

DURHAM, N.C.--(BUSINESS WIRE)--Mar. 10, 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 presentation of new preclinical study data supporting the potential long-term efficacy of PBGENE-DMD. The data presentation took place during a poster session at the Muscular Dystrophy Association (MDA) Clinical & Scientific Conference 2026 currently taking place in Orlando, Florida.

"As we prepare to commence the Phase1/2 FUNCTION-DMD clinical trial for PBGENE-DMD, we're excited to present new preclinical data highlighting its potential to deliver durable, long-term functional benefit through a differentiated *in vivo* gene editing approach designed to permanently correct the root cause of Duchenne muscular dystrophy (DMD)," said Cassie Gorsuch, PhD, Chief Scientific Officer at Precision BioSciences.

In new data from a GLP study conducted in a humanized DMD mouse model which replicates muscle degeneration observed in patients with DMD, PBGENE-DMD treatment led to improvements in numerous markers of muscle damage, including ALT (Alanine Aminotransferase), AST (Aspartate Aminotransferase), and CK (Creatine Kinase). At 90 days after administration of PBGENE-DMD, treatment resulted in a ~50-65% reduction in CK levels, indicating substantial muscle integrity improvements. Furthermore, results showed improvements in muscle pathology assessments with lower composite injury scores across multiple muscle tissues compared to control vehicle-treated mice. Together, these data support PBGENE-DMD's differentiated profile as a gene editing therapy designed to potentially treat up to 60% of DMD patients by restoring production of a near full-length, functional dystrophin protein through permanent gene correction.

As previously reported, PBGENE-DMD-treated mice showed significant and sustained improvements in muscle function over time, with treated mice maintaining approximately 81% to 84% of maximal force output and 89% to 92% of tetanic force output observed in healthy mice through nine months following treatment. The data further indicate that functional outcomes improved between three and six months and remained durable through the nine-month follow-up period. The functional data were supported by evidence of therapeutic levels of naturally expressed functional dystrophin protein in both skeletal and cardiac muscle, with dystrophin levels increasing over time through nine months. Broad and increasing levels of dystrophin-positive fibers were observed across key muscle groups, including the quadriceps, gastrocnemius, heart, and diaphragm, potentially driven by PBGENE-DMD edited muscle satellite stem cells, which were observed in the skeletal muscles of treated mice. Because satellite cells are essential for ongoing muscle regeneration in DMD, these findings reinforce PBGENE-DMD's potential as a one-time treatment designed for long-term benefit.

About PBGENE-DMD

PBGENE-DMD, a novel first-in-class gene editing therapy, utilizes a gene excision approach that is clearly differentiated from existing microdystrophin and exon skipping treatments. PBGENE-DMD is designed to potentially provide durable functional muscle improvement for DMD patients with mutations in exons 45-55, representing up to 60% of boys with DMD. A single AAV encodes two ARCUS proteins designed to permanently edit a patient's DNA within the dystrophin gene, resulting in a naturally-expressed, near full-length, functional dystrophin protein. Supported by robust preclinical evidence, PBGENE-DMD is designed to drive functional improvement over time by targeting muscle satellite cells.

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, diaphragm 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.

PBGENE-DMD has received FDA Fast Track, Rare Pediatric Disease, and Orphan Drug designations for the treatment of DMD. Following the IND clearance in early 2026, Precision is working with multiple institutional review boards and the FDA to initiate clinical site activations in the U.S. in the first half of 2026.

About FUNCTION-DMD Trial:

The Phase 1/2 FUNCTION-DMD study is expected to enroll ambulatory DMD patients between the age of 2-7 with mutations between exons 45 and 55 representing up to 60% of boys with DMD. The objective of the FUNCTION-DMD study is to evaluate safety, tolerability, and efficacy, including dystrophin protein expression and functional outcomes in patients afflicted with DMD. For more information about this clinical trial and contact information, please visit www.clinicaltrials.gov and search for NCT07429240.

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, expectations about operational initiatives, strategies, further development, clinical trial site activation, and therapeutic outcomes including potentially treating up to 60% of DMD patients by restoring production of a near full-length, functional dystrophin protein through permanent gene correction; translation of results in preclinical studies to clinical studies in humans; and the potential of PBGENE-DMD for durable dystrophin restoration across key muscle groups, durable efficacy and sustained functional improvement through satellite cell editing, and as a one-time treatment designed for long-term benefit. 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, 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; 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; our ability to advance product candidates into, and successfully design, implement and complete, clinical trials; changes in interim “top-line” and initial data that we announce or publish; our current and future relationships with and reliance on third parties including suppliers and manufacturers; 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 and our Quarterly Reports on Form 10-Q for the quarterly periods ended March 31, 2025, June 30, 2025, and September 30, 2025 as any such factors may be updated from time to time in our other filings with the U.S. Securities and Exchange Commission (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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