Precision BioSciences Receives U.S. FDA Clearance of Investigational New Drug Application for First-in-Class PBGENE-DMD for Treatment of Duchenne Muscular Dystrophy

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– U.S. FDA Study May Proceed notification enables initiation of clinical trial site activation for the FUNCTION-DMD Phase 1/2 clinical study in patients with Duchenne muscular dystrophy (DMD) –

– Institutional Review Board (IRB) process is underway at multiple world-class DMD clinical trial sites –

– PBGENE-DMD is an *in vivo* gene editing investigational product designed to correct the underlying genetic cause of DMD through a novel one-time gene editing therapy –

– Company plans to host a virtual event in March after the Muscular Dystrophy Association conference to discuss PBGENE-DMD and the FUNCTION-DMD clinical study with participation from key opinion leaders including patient advocacy –

DURHAM, N.C.--(BUSINESS WIRE)--Feb. 11, 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 that the Company received a Study May Proceed notification from the U.S. Food and Drug Administration (FDA). This allows Precision BioSciences to initiate IRB activities and clinical trial site activation for the FUNCTION-DMD Phase 1/2 clinical trial for PBGENE-DMD for the treatment of ambulatory Duchenne muscular dystrophy (DMD) patients at highly specialized clinical trial sites. The objective of the study is to evaluate safety, tolerability and efficacy, including dystrophin protein expression and functional outcomes in patients afflicted with DMD.

PBGENE-DMD is Precision's wholly-owned, first-in-class *in vivo* gene editing program utilizing a gene excision approach intended to permanently correct the dystrophin gene in patients with mutations between exons 45 and 55, the "hot-spot" region, which accounts for the largest molecular subset of patients with DMD at approximately 60%. The therapeutic goal is to restore the ability of the dystrophin gene to produce a near full-length functional dystrophin protein. This protein retains 80% of full-length dystrophin, substantially larger than synthetic micro-dystrophin constructs approved or in development today (which are approximately 34% of the size of full-length dystrophin).

"Duchenne muscular dystrophy is a progressive disorder caused by mutations in the dystrophin gene that disrupt the production of the functional protein, resulting in continuous muscle degeneration," said Aravindhan Veerapandiyan, M.D., Director, Comprehensive Neuromuscular Program, Arkansas Children's Hospital, University of Arkansas for Medical Sciences. "PBGENE-DMD is designed to restore near full-length dystrophin, with the potential to provide significant functional benefits. This is an important milestone, and I look forward to participating in the Phase 1/2 FUNCTION-DMD clinical study to evaluate a gene excision approach in DMD patients with mutations involving exons 45-55."

"PBGENE-DMD is an important step forward for the Duchenne community as it's the first ever gene editing approach designed to treat the majority of DMD patients. Families are urgently waiting for newer therapies that can meaningfully and durably change the course of this disease, and we are encouraged to see programs advancing that are designed to address the underlying genetic cause," said Pat Furlong, Founding President and CEO, Parent Project Muscular Dystrophy. "We look forward to learning more as the FUNCTION-DMD study begins and to continued collaboration between patient advocates, physicians and developers of new treatments to accelerate progress for every person living with Duchenne."

"The Study May Proceed notification for PBGENE-DMD by the FDA represents yet another regulatory achievement for Precision BioSciences as we advance our second wholly owned program toward the clinic. Despite approved therapies today, boys with DMD are lacking treatments that lead to functional improvements over time. We're excited to bring this novel gene excision approach for DMD to the clinic with the goal of activating the first clinical site in the U.S. in the first half of 2026. I'm proud of our team for meeting our goal of filing the IND by year end and receiving the FDA Study May Proceed notification in the first quarter. The Company will continue to work with multiple IRBs and the FDA to initiate clinical site activations," said Michael Amoroso, Chief Executive Officer of Precision BioSciences.

Upcoming Company Event

Precision plans to host a virtual investor event in March after the Muscular Dystrophy Association meeting discussing the PBGENE-DMD program and the planned Phase 1/2 FUNCTION-DMD clinical study. This event will include multiple KOLs from the medical and patient advocacy community. Further details to follow in a future press release.

About PBGENE-DMD

PBGENE-DMD, a novel first-in-class gene editing therapy, utilizes a gene excision approach, which 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 impacting 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 in skeletal and cardiac muscle over time with the ability to target and edit 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 production of 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.

PBGENE-DMD was recently granted FDA Rare Pediatric Disease (RPD) and Orphan Drug (ODD) designation for the treatment of DMD.

About FUNCTION-DMD Trial:

The Phase 1/2 FUNCTION-DMD study is expected to enroll ambulatory DMD patients with mutations in exons 45-55 impacting up to 60% of boys with DMD. The clinical trial will employ an appropriate immune modulation regimen and safety monitoring program to treat ambulatory patients with mutations in exons 45-55 at world class specialized DMD clinical sites. Initial data from multiple patients is expected by year end 2026, with early efficacy assessed by the percentage of near full-length dystrophin protein expression from muscle biopsies. Following supportive data from at least 10 DMD patients, the company would meet with the FDA to align on a regulatory path forward.

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 including dose expansion, and timing of additional updates or data releases of PBGENE-DMD and the FUNCTION-DMD Trial; translation of results in preclinical studies of PBGENE-DMD to clinical studies in humans; expected timing and outcome of regulatory and institutional review board processes for PBGENE-DMD; the design of PBGENE-DMD to restore near full-length dystrophin, with the potential to confer meaningful functional benefits; the design of PBGENE-DMD to correct the underlying genetic cause of DMD and restore near full length dystrophin through a novel one-time gene editing treatment approach; initiation of the Institutional Review Board review process to enable site activation in the first half of 2026; continued work with multiple IRBs and the FDA to initiate clinical site activations; and the Company's plans to host a virtual event for investors and interested stakeholders to discuss the PBGENE-DMD program, IND clearance, and the planned Phase 1/2 FUNCTION-DMD clinical study. 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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