



Precision BioSciences Activates First Clinical Trial Site and Begins Patient Enrollment in Phase 1/2 FUNCTION-DMD Study

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- Arkansas Children's Hospital activated as the first clinical trial site and now enrolling patients in the FUNCTION-DMD study of PBGENE-DMD -

DURHAM, N.C.--(BUSINESS WIRE)--Apr. 29, 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 the activation of the first clinical trial site and the opening of patient screening and enrollment for PBGENE-DMD. PBGENE-DMD, the first-in-class *in vivo* gene editing treatment for Duchenne muscular dystrophy (DMD), is being evaluated in the Phase 1/2 FUNCTION-DMD study.

PBGENE-DMD is Precision's wholly owned *in vivo* gene editing program designed to potentially provide durable functional muscle improvement for patients with DMD who have mutations between exons 45 and 55, the largest molecular subset of boys living with the disease. The Phase 1/2 FUNCTION-DMD study will enroll ambulatory patients and is designed to evaluate safety, tolerability, and efficacy, including dystrophin protein expression and functional outcomes.

"The activation of Arkansas Children's Hospital marks a critical step in advancing PBGENE-DMD into the clinic and expanding access for patients and families who are looking for novel options to address this devastating genetic disease," said Michael Amoroso, Chief Executive Officer of Precision BioSciences. "Dr. Aravindhan Veerapandiyam and the team at Arkansas Children's Hospital bring deep experience in running clinical trials and research in Duchenne. As the inaugural site for the FUNCTION-DMD study, we are pleased to open screening and enrollment at this center while we continue to work to activate other trial sites for inclusion in the FUNCTION-DMD study."

Arkansas Children's is a Parent Project Muscular Dystrophy (PPMD)-certified Duchenne Care Center, recognized for delivering specialized, multidisciplinary care for patients with Duchenne muscular dystrophy. PPMD's Certified Duchenne Care Center Program is intended to help ensure that participating centers maintain high standards in clinical and sub-specialty services, rapidly incorporate evidence-based knowledge, and provide standardized multidisciplinary Duchenne care. Additionally, Arkansas Children's is a designated Muscular Dystrophy Association (MDA) Care Center, providing specialized, multidisciplinary care for neuromuscular diseases including diagnosis, personalized treatment plans, and comprehensive support for patients.

"Families living with Duchenne Muscular Dystrophy need access to both experienced multidisciplinary care and promising investigational studies that may address the underlying cause of disease," said Aravindhan Veerapandiyam, M.D. "We are pleased to begin screening and enrollment for the FUNCTION-DMD study in patients from around the world who seek treatment at our site and look forward to working with Precision and eligible patients and families as we evaluate PBGENE-DMD."

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.

About PBGENE-DMD, A Muscle-Targeted Excision Program

PBGENE-DMD is Precision's development program for the treatment of DMD. 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 durable and significant functional improvements over time. PBGENE-DMD is designed to improve function by employing two complementary ARCUS nucleases delivered in a single AAV to excise exons 45-55 of the dystrophin gene. The aim of this approach is to restore a near full-length functional dystrophin protein within the body that more closely resembles normal dystrophin as opposed to synthetic, truncated microdystrophin approaches with potentially minimal functional benefit. The Phase 1/2 FUNCTION-DMD study is expected to enroll ambulatory DMD patients with mutations between exons 45 and 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 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.

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 expectations around timing of site activation, patient enrollment and data releases of PBGENE-DMD and the FUNCTION-DMD Trial; translation of results in preclinical studies of PBGENE-DMD to clinical studies in humans; the design of PBGENE-DMD to

potentially provide durable functional muscle improvement for DMD patients with mutations in exons 45-55 impacting up to 60% of patients with DMD; the belief that activation at a center with a high level of specialized expertise is important to advance a novel therapy; the use of an appropriate immune modulation regimen; and the design of PBGENE-DMD, a first-in-class gene editing approach designed to address the underlying cause of Duchenne muscular dystrophy. 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-K for the annual period ended December 31, 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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