Precision BioSciences Presents Preclinical Efficacy and Durability Data on PBGENE-DMD for the Treatment of Duchenne Muscular Dystrophy (DMD) at the 2025 Muscular Dystrophy Association (MDA) Clinical & Scientific Conference

March 19, 2025 at 7:01 AM EDT

- Potential first-in-class gene editing approach designed for dystrophin gene correction leading the body to produce a functional dystrophin protein applicable for majority of DMD patients (up to ~60%)

- PBGENE-DMD restored dystrophin protein expression and significantly improved muscle function over time while demonstrating long-term durability in an in vivo DMD disease model –

- PBGENE-DMD dystrophin gene correction observed in muscle satellite stem cells suggesting potential for permanent functional benefit –

DURHAM, N.C.--(BUSINESS WIRE)--Mar. 19, 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, including novel gene excision programs for high unmet need genetic diseases, today announced the presentation of preclinical data for its PBGENE-DMD development program for the treatment of Duchenne muscular dystrophy (DMD) during an oral presentation at the 2025 Muscular Dystrophy Association (MDA) Clinical & Scientific Conference being held March 16-19, 2025 in Dallas, TX.

"While there has been much-needed progress in the DMD field recently, patients still lack treatments that offer significant durable functional improvement. These PBGENE-DMD preclinical data compellingly demonstrate the potential for gene correction in the body to natively produce near full length dystrophin and restore muscle function while offering durability through the editing of muscle satellite stem cells," said Dr. Cassie Gorsuch PhD, Chief Scientific Officer. "By precisely and directly excising the genetic root cause for DMD patients with defects between exon 45 and 55, our approach could provide more durable outcomes for these patients compared to microdystrophin gene therapies. Furthermore, this therapeutic approach is applicable for up to 60% of DMD patients, far more than exon skipping approaches currently approved or in development. The results presented today demonstrate the therapeutic potential of PBGENE-DMD to improve the lives of patients with DMD and support future clinical development of the first widely applicable gene editing approach."

Presentation Details:

Title: ARCUS-Mediated Excision of Exons 45-55 Leads to Functional Del45-55 Dystrophin and Restoration of Skeletal Muscle-Function for the Treatment of DMD
Oral Presentation Date and Time: Wednesday, March 19, 2025, 8:00 AM CT
Poster Number: O159

In preclinical data to be presented today, PBGENE-DMD demonstrated significant functional improvement in a humanized DMD mouse model by employing two complementary ARCUS nucleases delivered in a single AAV to excise exons 45-55 of the dystrophin gene. This approach aims to restore the body's native production of a functional dystrophin protein that more closely resembles normal dystrophin than synthetic microdystrophins. This dystrophin gene correction approach which involves editing muscle satellite stem cells potentially enhances durability and functional outcomes compared to synthetic approaches. Since up to 60% of DMD cases are caused by defects between exons 45 and 55, this approach is more broadly applicable for the majority of DMD patients than exon skippers.

Key findings from the study include:

- **Functional dystrophin protein production**: PBGENE-DMD restored the body's ability to produce a functional dystrophin protein across multiple muscles, including heart, diaphragm, and skeletal muscles at levels expected to provide therapeutic benefit.
- **Enhanced Muscle Resilience:** Treated mice exhibited a 66% improvement in resistance to eccentric injury, an indicator of enhanced muscle resilience, compared to untreated diseased counterparts.
- **Long-Term Functional Improvement:** In mice treated with PBGENE-DMD the maximum force output (MFO), a critical functional metric, reached up to 93% of the MFO in healthy control mice with improvement observed in PBGENE-DMD-treated mice between 3 and 6 months.
- **Durable Outcomes**: PBGENE-DMD-edited dystrophin mRNA transcript was detected in PAX7+ cells, a marker for muscle satellite stem cells, suggesting potential for durable therapeutic effects compared to standard gene therapy approaches.

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 or mutant mitochondrial DNA), and excision (removing a large portion of a defective gene by delivering two ARCUS nucleases in a single AAV like in the DMD program).

About Duchenne Muscular Dystrophy (DMD)

DMD is a genetic disease caused by mutations in the dystrophin gene that prevent production of the dystrophin protein. Dystrophin stabilizes the cell membrane during muscle contraction to prevent damage, and the absence of intact dystrophin protein leads to inflammation, fibrosis, and progressive loss of muscle function and mass. Over time, children with DMD will develop problems walking and breathing, eventually leading to death in their second or third decade of life due to progressive cardiomyopathy and respiratory insufficiency. DMD occurs in 1 in 3,500 to 5,000 male births with an estimated prevalence of 15,000 patients and incidence of 550 patients/year in the United States alone. Unmet need for DMD patients remains high as there are no approved therapies with curative intent that can drive durable and significant functional improvements.

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 clinical development and expected safety, efficacy and benefit of our product candidates (including PBGENE-DMD); the unique design of PBGENE-DMD utilizing two ARCUS nucleases delivered using a single AAV to restore native function to the dystrophin protein by excising exons 45-55 as a treatment; the suitability of PBGENE-DMD for the treatment of Duchenne Muscular Dystrophy by directly editing the genetic root cause of the disease to potentially provide more durable outcomes for the majority of patients compared to gene therapies and exon skippers currently approved or in development; the potential of PBGENE-DMD as a first-in-class gene editing approach targeting up to 60% of DMD patients; the expected timing of regulatory processes (including filings such as IND's and CTA's and studies for PBGENE-DMD and the acceptance of these filings by regulatory agencies); the robust safety, tolerability and efficacy signals observed through preclinical evaluation in transgenic mouse models, human cell models of DMD; the translatability of preclinical models to human clinical trials; the key advantages of ARCUS and its key capabilities and differentiating characteristics ; expectations about operational initiatives, strategies, and further development of PBGENE-DMD; expectations about achievement of key milestones; the assessment of whether to partner or internally develop the PBGENE-DMD clinical program; and anticipated timing of patient dosing and clinical data. 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,", "suggest", "strive," "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 September 30, 2024, 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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