Precision BioSciences Announces Multiple Presentations for In Vivo Gene Editing Programs at the American Society of Gene and Cell Therapy (ASGCT) Annual Meeting

May 1, 2025 at 7:01 AM EDT

DURHAM, N.C.--(BUSINESS WIRE)--May 1, 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 gene elimination, gene insertion, and gene excision programs, today announced that five abstracts detailing Precision's wholly owned or partnered programs have been accepted for presentation at the American Society of Gene and Cell Therapy (ASGCT) Annual Meeting being held May 13-17, 2025, in New Orleans, Louisiana.

"We are pleased to present new data at ASGCT from multiple programs across our *in vivo* gene editing pipeline, highlighting the potentially unmatched breadth and therapeutic potential of our ARCUS gene editing technology," said Michael Amoroso, President and Chief Executive Officer at Precision BioSciences. "Clinically, we have made progress advancing our lead program, PBGENE-HBV, which is a novel gene editing approach being evaluated in a first-in-human study for chronic hepatitis B, and we look forward to sharing initial safety data from the first two dose cohorts at ASGCT. Additionally, we continue to be excited by the promising clinical results generated thus far by our partner iECURE for ECUR-506 in OTC deficiency, which utilizes an ARCUS nuclease and will also be presenting data at this conference."

"We are also excited to share promising preclinical results from two muscle programs, PBGENE-3243 program for m.3243-associated mitochondrial disease and PBGENE-DMD for the treatment of Duchenne Muscular Dystrophy (DMD)," Mr. Amoroso continued. "Collectively, these presentations highlight the progress we are making with ARCUS by deploying a variety of gene edit types, including gene elimination, gene insertion and gene excision across a range of complex genetic diseases as we seek to develop durable, curative treatments for patients."

Clinical Program Presentations:

Title: Initial Safety Data From ELIMINATE-B, the First Clinical Trial of a Gene Editing Treatment for Chronic Hepatitis B
Oral Presentation Session: Gene Therapy Trials - In-Vivo Gene Therapy Modification
Date and Time: Friday, May 16, 2025, 4:00 PM CT
Location: Room 393-396

Title: ARCUS *in vivo* OTC, Large Gene Insertion *(clinical stage partnered program)*
Oral Presentation Session: Advances in Genome Editing: Novel Large DNA Insertion Technologies and Their Potential Towards Curative Therapies
Date and Time: Wednesday, May 14, 2025, 8:00 AM CT
Location: NOLA Theater B

Preclinical Program Presentations:

Title: Excision of the *C9orf72* Hexanucleotide Repeat Expansion Using a Dual-ARCUS Gene Editing Approach Reduces Neurotoxic RNA Foci and Dipeptides in an *In Vivo* Model of ALS
Poster Session: Poster Reception
Date and Time: Tuesday, May 13, 2025, 6:00 PM - 7:30 PM CT
Location: Poster Hall I2

Title: Systemic Delivery of a Mitochondria-Targeting ARCUS Gene Editing Nuclease by AAV Eliminates Mutant Mitochondrial DNA, Demonstrating Therapeutically Meaningful Heteroplasmy Shifts In Vivo
Oral Presentation Session: Gene Editing: New Tools and Technology Advances
Date and Time: Wednesday, May 14, 2025, 3:45 PM CT
Location: NOLA Theater A

Title: ARCUS-Mediated Gene Editing Excision of Exons 45-55 of the Human Dystrophin Gene using PBGENE-DMD Leads to Functional Dystrophin Protein and Durable Restoration of Skeletal Muscle-Function In Vivo for the Treatment of Duchenne Muscular Dystrophy
Poster Session: Poster Reception
Date and Time: Wednesday, May 14, 2025, 5:30 PM - 7:00 PM CT
Location: Poster Hall I2

The abstracts are now publicly accessible through the ASGCT website here.

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 such as in the DMD program).

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 therapeutic potential of ARCUS compared to other gene editing technologies; clinical development and expected safety, efficacy and benefit of our wholly owned gene editing product candidates PBGENE-HBV, PBGENE-DMD, PBGENE-3243, and PBGENE-CNS programs and those being advanced by our collaborators and partners including ECUR-506; our gene editing approaches including editing efficiency, and the suitability of ARCUS nucleases for gene insertion, gene elimination and gene excision and differentiation from other gene editing approaches; the expected timing of regulatory processes and clinical operations for our wholly-owned and partnered product candidates, including filings, studies, enrollment and clinical data; the translatability of preclinical data to development of durable, curative treatments for patients; the initial safety and antiviral activity observed in the ELIMINATE-B clinical trial; plans to provide ongoing updates on the PBGENE-HBV study; anticipated timing of clinical data; clinical efficacy and safety data in the OTC-HOPE clinical trial; ability of an ARCUS nuclease to eliminate mutant mitochondrial DNA and achieve therapeutically meaningful heteroplasmy shifts *in vivo*; the therapeutic potential of PBGENE-DMD and advancement through preclinical studies; and the potential of proof of concept for ARCUS-mediated excision of the *C9orf72* gene HRE and further development of PBGENE-CNS for ALS. In some cases, you can identify forward-looking statements by terms such as “aim,” “anticipate,” “appear,” “approach,” “believe,” “contemplate,” “could,” “designed,” “encouraged”, “estimate,” “expect,” “goal,” “intend,” “look,” “may,” “mission,” “plan,” “possible,” “potential,” “predict,” “project,” “promising,” “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, but involve 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 raising additional capital and requirements under our current debt instruments and effects of restrictions thereunder; our operating expenses and our ability to predict what those expenses will be; our limited operating history; the 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, preclinical studies and clinical trials; public perception about genome editing technology and its applications; competition in the genome editing, biopharmaceutical, and biotechnology fields; our or our collaborators’ ability to identify, develop and commercialize product candidates; potential product liability lawsuits and penalties against us or our collaborators related to our technology and our product candidates; the U.S. and foreign regulatory landscape applicable to our and our collaborators’ development of product candidates; our or our collaborators’ or other licensees’ ability to advance product candidates into, and successfully design, implement and complete, clinical or field trials; potential manufacturing problems associated with the development or commercialization of any of our product candidates; delays or difficulties in our and our collaborators’ 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 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’ 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 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 Annual Report on Form 10-K for the annual period ended December 31, 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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Investor and Media Contact:
Naresh Tanna
Vice President of Investor Relations
naresh.tanna@precisionbiosciences.com

Source: Precision BioSciences, Inc.