Precision BioSciences Reports First Quarter 2025 Financial Results and Provides Business Update

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- Announced new clinical data for two programs utilizing ARCUS validating safety and efficacy for Precision's in vivo gene editing approach

- Announced initial data from first cohort in the ongoing Phase 1 ELIMINATE-B trial evaluating PBGENE-HBV; initial safety data presented at EASL; ongoing clinical updates anticipated throughout 2025

- Received regulatory clearance of IND from the U.S. FDA to expand ELIMINATE-B trial and CTA approved by MHRA for study expansion into the UK. Additionally, PBGENE-HBV granted U.S. FDA Fast Track Designation for chronic Hepatitis B (HBV)

- Accelerating PBGENE-DMD within Precision pipeline to be first-in-class in vivo gene editing approach addressing majority of Duchenne's Muscular dystrophy (DMD) patients; targeting to file IND/CTA in 2025 with clinical data in 2026

- Expected cash runway into second half of 2026 enabling data read outs from first two wholly owned in vivo gene editing programs – PBGENE-HBV and PBGENE-DMD

DURHAM, N.C.--(BUSINESS WIRE)--May 15, 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 for diseases with high unmet need, today announced financial results for the first quarter ended March 31, 2025, and provided a business update.

"We started 2025 with strong momentum and a focus on generating impactful clinical data across our *in vivo* gene editing pipeline. Early this year, data from the OTC-HOPE trial provided the first clinical validation for ARCUS *in vivo* gene insertion following a complete response in an infant with OTC-deficiency," said Michael Amoroso, Chief Executive Officer of Precision BioSciences. "In addition, the Phase 1 ELIMINATE-B trial evaluating PBGENE-HBV, our lead program for chronic Hepatitis B, is progressing as planned. We presented encouraging data at the recent EASL Congress highlighting the safety of PBGENE-HBV after repeat dosing as planned in the ELIMINATE-B protocol. We look forward to sharing additional updates from the ELIMINATE-B trial throughout 2025, including antiviral efficacy data from each cohort upon completion of three dose administrations. In addition, we recently received U.S. investigational new drug (IND) approval to expand this important global trial as well as Fast Track Designation from the U.S. Food and Drug Administration (FDA) underscoring ARCUS' potential to deliver a curative treatment option for people in need living with chronic Hepatitis B."

"In tandem with the ongoing clinical trials, we continued to advance our *in vivo* gene editing programs and shared encouraging data across multiple assets at ASGCT. Of note, we presented exciting proof of concept data from PBGENE-DMD. This data was the first pre-clinical proof of protein expression linked to improved and sustained muscle function across 3-, 6-, and 9-month time points in disease models. PBGENE-DMD makes a correction in the body's dystrophin gene to produce a near full length functional dystrophin that is present in nature. Due to its targeting of satellite muscle stem cells which are the progenitor cells for new muscle cells, PBGENE-DMD's one-time correction approach aims to offer the potential for durable functional improvement with lower doses of AAV. In consideration of the data, the high unmet need of those afflicted with DMD due to limited treatment options, and the established regulatory support and guidance within the FDA, we have prioritized this program to be our second wholly owned program with a target IND and/or clinical trial application (CTA) in 2025," continued Mr. Amoroso. "This reinforces our commitment to delivering transformative therapies in genetic diseases with the highest unmet need and driving near and long-term value while being prudent stewards of capital."

Wholly Owned Portfolio

PBGENE-HBV (Viral Elimination Program): PBGENE-HBV is Precision's wholly owned *in vivo* gene editing program under investigation in a global first-in-human clinical trial, which is designed to be a potentially curative treatment for chronic Hepatitis B infection. PBGENE-HBV is the first and only potentially curative gene editing program to enter the clinic that is specifically designed to eliminate cccDNA and inactivate integrated HBV DNA, the root cause chronic Hepatitis B. The ELIMINATE-B trial is investigating PBGENE-HBV at multiple ascending dose levels with three dose administrations per dose level in patients with chronic Hepatitis B.

In February 2025, Precision announced initial results from the first administration of PBGENE-HBV in cohort 1, the lowest dose level of the ELIMINATE-B trial. PBGENE-HBV was safe and well tolerated in all three participants in cohort 1 after the first administration of a 0.2 mg/kg dose. In addition to safety, PBGENE-HBV demonstrated a substantial reduction in Hepatitis B surface antigen (HBsAg) in two of the three participants following the first administration at the lowest dose level.

In March 2025, the FDA cleared PBGENE-HBV to commence Phase 1 clinical trials in the U.S and in April 2025, the FDA granted Fast Track designation to PBGENE-HBV for chronic Hepatitis B. Throughout 2025, the Company plans to share updates on full cohorts including antiviral efficacy at different dose levels as each cohort is completed following three dose administrations and appropriate follow up. Also, in April 2025, the U.K. Medicines and Healthcare products Regulatory Agency cleared PGENE-HBV for Phase 1 trial making it the fifth country to clear a CTA or IND for PBGENE-HBV.

On May 8, 2025, Precision presented initial safety data for PBGENE-HBV at the European Association for the Study of the Liver Congress (EASL), which indicated translation of PBGENE-HBV nonclinical pharmacokinetic and safety data from non-human primates into the clinic. These data support

pre-planned repeat dosing as well as dose escalation of PBGENE-HBV with the goal of moving appropriate dose and schedule into Phase 2 expansion.

PBGENE-DMD (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 over time and addresses more than 60% of patients afflicted with DMD by employing two complementary ARCUS nucleases delivered in a single AAV to excise exons 45-55 of the dystrophin gene with the aim of restoring a near-full length functional dystrophin protein within the body that more closely resembles normal dystrophin as opposed to synthetic, truncated dystrophin approaches with minimal functional benefit.

In preclinical data presented at the muscular Dystrophy Association (MDA) in March 2025 and additional new data presented at the American Society of Gene and Cell Therapy (ASGCT) on May 14, 2025, PBGENE-DMD demonstrated the ability to target key muscle types involved in the progression of DMD and significant, durable functional improvement 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 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.

Based on these compelling data, the significant unmet need in DMD, and the clear regulatory guidance established for new therapeutics for DMD, Precision believes that PBGENE-DMD holds significant potential for the majority of patients with DMD. As a result, Precision is prioritizing and accelerating the development of PBGENE-DMD as its second wholly owned clinical program and targets filing an IND and/or CTA for PBGENE-DMD in 2025 with clinical data expected in 2026.

PBGENE-3243 (Mutant Mitochondrial DNA Elimination Program): PBGENE-3243 is a first-of-its-kind potential treatment for m.3243-associated mitochondrial disease that is designed to specifically target and eliminate mutant m.3243G mitochondrial DNA, thereby eliminating the root cause of the disease. Currently, there are no curative treatments for m.3243-associated mitochondrial disease, which affects approximately 20,000 people in the U.S. alone and an even larger prevalent population globally. PBGENE-3243 is designed to alleviate muscular myopathy symptoms, providing a significant quality of life and functional improvement for patients.

New preclinical data presented at ASGCT on May 14, 2025, highlight the ability of an ARCUS nuclease to eliminate mutant mitochondrial DNA and achieve therapeutically meaningful heteroplasmy shifts *in vivo*. Reductions of mutant mtDNA exceed the baseline cited in the literature to be sufficient to alleviate the clinical symptoms in patients with m.3243-associated disease.

To accelerate development of PBGENE-DMD and maintain operational capability to deliver PBGENE-HBV and PBGENE-DMD data through Phase 1 clinical results, Precision plans to pause development of PBGENE-3243 and will stage future development of PBGENE-3243 alone or with partners following completion of the Phase 1 ELIMINATE-B HBV trial and after the PBGENE-DMD program reaches the clinic.

Partnered Programs:

iECURE-OTC (Gene Insertion Program): Led by iECURE, ECUR-506 is an ARCUS-mediated *in vivo* gene editing program currently in a first-in-human Phase 1/2 trial (OTC-HOPE) evaluating ECUR-506 as a potential treatment for neonatal onset ornithine transcarbamylase (OTC) deficiency. In January 2025, iECURE reported clinical efficacy and safety data in the first patient dosed showing a complete clinical response from three months post-exposure to the end of study at six months, as defined by the study protocol. The patient is now more than one year old and is eating age-appropriate levels of protein for a child of this age. Clinical data from the first patient in the ongoing OTC-HOPE trial was presented at the 2025 ACMG Annual Clinical Genetics Meeting in March 2025 and additional data has been accepted for presentation at ASGCT 2025.

The OTC-HOPE study is ongoing in the U.K., the U.S., Australia, and Spain, and iECURE expects to complete enrollment in 2025 and anticipates complete data from the trial in the first half of 2026.

PBGENE-NVS (Gene Insertion Program): Precision continues to advance its gene editing program with Novartis to develop a custom ARCUS nuclease for patients with hemoglobinopathies, such as sickle cell disease and beta thalassemia. The collaborative intent is to insert, *in vivo*, a therapeutic transgene as a potential one-time transformative treatment administered directly to the patient to overcome disparities in patient access to treatment with other therapeutic technologies, including those that are targeting an ex vivo gene editing approach.

Quarter Ended March 31, 2025 Financial Results:

Cash, Cash Equivalents, and Restricted Cash: As of March 31, 2025, Precision had approximately $100 million in cash, cash equivalents, and restricted cash. The Company expects that existing cash and cash equivalents, potential near-term cash from CAR T transactions, along with expected operational receipts, continued fiscal and operating discipline, and availability of Precision's at-the-market (ATM) facility to extend Precision's cash runway into the second half of 2026. Based on its expected cash runway, Precision believes it is sufficiently capitalized to advance its two lead wholly owned programs, PBGENE-HBV and PBGENE-DMD through Phase 1 data readouts.

Revenues: Total revenues for the quarter ended March 31, 2025, were less than $0.1 million, as compared to $17.6 million for the quarter ended March 31, 2024. The decrease of $17.6 million was due to revenue recognized from TG Therapeutics and Caribou Biosciences agreements in the quarter ended March 31, 2024, a decrease in the revenue recognized under the Prevail agreement following conclusion of the collaboration in April 2024, and a decrease in revenue recognized under the Novartis Agreement as Precision nears completion of its pre-clinical workplan.

Research and Development Expenses: Research and development expenses were $13.6 million for the quarter ended March 31, 2025, as compared to $13.3 million for the quarter ended March 31, 2024. The increase of $0.3 million was primarily due to an increase in direct expense for PBGENE-DMD as the program advances towards the clinic.

General and Administrative Expenses: General and administrative expenses were $8.6 million for the quarter ended March 31, 2025, as compared to $8.4 million for the quarter ended March 31, 2024. The increase of $0.2 million was primarily due to employee-related costs, partially offset by decreases in depreciation and amortization expense as well as taxes and insurance.

Net Loss: Net loss was $20.6 million, or $(2.21) per share (basic and diluted), for the quarter ended March 31, 2025. Net income was $8.6 million, or $1.70 per share (basic and diluted), for the quarter ended March 31, 2024.

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. Precision's two lead programs, PBGENE-HBV, for chronic Hepatitis B, and PBGENE-DMD, for Duchenne muscular dystrophy are focused on areas with large patient populations with high unmet need. 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 a gene to cause expression/add function), elimination (removing a genome e.g. viral DNA), and excision (removing a large portion of a defective gene by delivering two ARCUS nucleases in a single AAV).

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 potential of PBGENE-DMD to be a first-in-class *in vivo* gene editing approach addressing the majority of DMD patients; the safety data and antiviral activity established after the administrations of PBGENE-HBV; translation of results in preclinical studies of ARCUS nucleases to clinical studies in humans; the preclinical and clinical development and demonstrated, potential and expected safety, efficacy and benefit of PBGENE-HBV and PBGENE-DMD, as well as our other product candidates and those being developed by partners; the unique design of PBGENE-HBV to eliminate cccDNA and inactivate integrated HBV DNA with high specificity, potentially leading to functional cures; the expected timing and opportunities of regulatory processes (including filings such as INDs or CTAs for PBGENE-HBV and PBGENE-DMD and the acceptance of these filings by regulatory agencies); the suitability of PBGENE-HBV for the treatment of hepatitis and the targeting of the root cause of the disease; the key advantages of ARCUS and its key capabilities and differentiating characteristics; expectations about operational initiatives, strategies, further development, or timing of additional updates or data releases of PBGENE-HBV and PBGENE-DMD; plans to provide additional administrations of PBGENE-HBV at the first dose level; plans to escalate to higher dose levels and next cohorts in the ELIMINATE-B clinical trial; expansion of the ELIMINATE-B clinical trial to the United States and United Kingdom; expectations around accelerating the PBGENE-DMD program; the design of PBGENE-DMD to improve function over time and address more than 60% of patients with DMD; the potential for PBGENE-DMD to provide durable functional improvement with a one time lower dose of AAV; the complete enrollment of the OTC-HOPE study in the U.S., the U.K., Australia, and Spain and timing of full data from the trial in the first half of 2026; expectations and announcements about achievement of key milestones; our expected cash runway and the sufficiency of our cash runway extending into the second half of 2026 to advance PBGENE-HBV and PBGENE-DMD through Phase 1 data readouts; and the staged development of PBGENE-3243 alone or with partners following completion of the Phase 1 ELIMINATE-B trial and after the PBGENE-DMD program reaches the clinic. 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," "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-Q for the quarterly period ended March 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.

Precision Biosciences, Inc.
Statements of Operations
(In thousands, except share and per share amounts)

	For the Three Months Ended March 31,	
	2025	**2024**
Revenue	$ 29	$ 17,584
Operating expenses		
Research and development	13,588	13,343
General and administrative	8,553	8,428
Total operating expenses	22,141	21,771
Operating loss	(22,112)	(4,187)
Other income (expense):		
Gain from equity method investment	1,342	1,713
Gain (loss) on changes in fair value	49	(348)
(Loss) gain on change in fair value of warrant liability	(804)	10,386
Interest expense	(354)	(574)
Interest income	1,323	1,663
Loss on disposal of assets	(9)	(65)
Total other income	1,547	12,775
(Loss) income from continuing operations	$ (20,565)	$ 8,588
Net (loss) income	$ (20,565)	$ 8,588
Net (loss) income per share		
Basic	$ (2.21)	$ 1.70
Diluted	$ (2.21)	$ 1.70
Weighted-average shares of common stock outstanding		
Basic	9,292,066	5,060,978
Diluted	9,292,066	5,063,406

Precision Biosciences, Inc.
Balance Sheets Data
(In thousands, except share amounts)

	March 31, 2025	**December 31, 2024**

Cash, cash equivalents, and restricted cash	$	99,789	$	108,468
Working capital		69,710		80,009
Total assets		124,411		136,388
Total liabilities		75,074		79,995
Total stockholders' equity	$	49,337	$	56,393
Common stock outstanding		10,548,852		8,202,715

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