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

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- Continued rapid progress with Phase 1 ELIMINATE-B trial evaluating PBGENE-HBV for Hepatitis B; Announced safety and efficacy for Cohort 1 and safety data for Cohort 2

- PBGENE-HBV demonstrated substantial antiviral activity in all Cohort 1 patients, with best responses achieving a 47-69% reduction in Hepatitis B surface antigen (HBsAg) and durable HBsAg reduction in one patient 7 months after initial dose administration

- Accelerated development of PBGENE-DMD as a potential first-in-class in vivo gene editing approach for Duchenne Muscular Dystrophy (DMD)

- Granted FDA Rare Pediatric Disease Designation and Orphan Drug Designation for PBGENE-DMD for the treatment of DMD; Clinical data expected in 2026.

- Extended expected cash runway to the second half of 2027 providing more than two years of operating cash

DURHAM, N.C.--(BUSINESS WIRE)--Aug. 7, 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 second quarter ended June 30, 2025, and provided a business update.

“Our team continues to be very disciplined about executing our plans and is making strong progress advancing our clinical stage PBGENE-HBV program while rapidly advancing PBGENE-DMD toward the clinic,” said Michael Amoroso, Chief Executive Officer of Precision BioSciences. “The early Phase 1 safety and efficacy data for PBGENE-HBV from the first cohort of the Phase 1 ELIMINATE-B trial establishes proof of activity for our novel gene editing approach for chronic Hepatitis B. Our data shows that we have a novel, safe and active drug in all patients treated with a durable effect in one third of patients reinforcing the mechanism of PBGENE-HBV to eliminate cccDNA. We are very pleased with the safety profile demonstrated in Cohorts 1 and 2 which has enabled the Data Monitoring Committee to endorse enrolling Cohort 3 this month to test the next higher dose. Concurrently, we are accelerating the development of our second program, PBGENE-DMD, and were proud to receive both Rare Pediatric Disease and Orphan Drug designations from the U.S. Food and Drug Administration (FDA), underscoring the significant unmet need for new therapeutic options for patients living with DMD.”

“Given the unmet need, opportunity and enthusiasm for PBGENE-HBV and PBGENE-DMD, we are taking proactive steps to invest fully in these two programs while extending our expected cash runway to the second half of 2027 through a significant reduction in our non-program related annual operating expenses. These actions are expected to enable commencement of a Phase 2 study for PBGENE-HBV and a potential pivotal trial for PBGENE-DMD. Our team remains committed to delivering transformative therapies in areas with significant unmet need and, with a longer cash runway, we believe we are now even better positioned to deliver the meaningful clinical data that is expected by patients and shareholders for both of our wholly-owned programs,” added Mr. Amoroso.

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 the root cause of chronic Hepatitis B, cccDNA, while inactivating integrated HBV DNA. 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.

On August 6, 2025, Precision announced Phase 1 safety and efficacy data for Cohort 1, the lowest dose level in the ELIMINATE-B trial. Cohort 1 consisted of three patients each of whom received three planned administrations of 0.2 mg/kg of PBGENE-HBV dosed approximately eight weeks apart. The primary objective is to characterize the safety of PBGENE-HBV. PBGENE-HBV was well-tolerated in all three patients in Cohort 1. Across Cohort 1, no patient experienced above a Grade 2 treatment-related adverse event, a serious adverse event, or dose-limiting toxicity. No clinically significant lab abnormalities were observed, including liver enzymes and platelets.

PBGENE-HBV demonstrated a substantial HBsAg reduction in all three patients in Cohort 1 with best response reductions of 56%, 69% and 47% compared to baseline HBsAg levels (ranging between 562-11,813 IU/mL) in patients one, two and three, respectively. One of three patients (33%) in Cohort 1 achieved a durable HBsAg reduction of approximately 50% from baseline that was maintained as of the data cutoff-date (July 28, 2025), which was seven months after initial dosing. This data provides evidence of the ability of PBGENE-HBV to drive a durable antiviral response by editing the viral DNA at the source of chronic Hepatitis B infection. There was no association between baseline HBsAg and efficacy in Cohort 1.

Precision also announced initial safety data from Cohort 2 (0.4 mg/kg) in the ELIMINATE-B study. As of the data cutoff, one patient received three dose administrations with two weeks of follow-up, and two patients received one dose administration with four weeks of follow-up. In these patients, no adverse events above Grade 2 were observed. No serious adverse events or dose limiting toxicities were observed, and no cumulative adverse effects were observed. There were no clinically significant elevations of liver transaminases. One additional patient did not complete their dose due to a

transient infusion-related serious adverse event that resolved within minutes. The Data Monitoring Committee determined that this transient reaction was not dose-related or dose-limiting. Given the favorable safety profile of Cohorts 1 and 2, the Data Monitoring Committee recently recommended initiation of Cohort 3.

The Company is on track to complete dosing of all three patients across all dose administrations in Cohort 2 and commence dosing Cohort 3. The Company expects to provide a data update later in 2025.

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 for 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. 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 dystrophin approaches with minimal functional benefit.

The FDA granted PBGENE-DMD Rare Pediatric Disease designation in June 2025 for the treatment of DMD, highlighting the significant unmet need for new therapeutic options. This was followed in July 2025 by the FDA granting PBGENE-DMD Orphan Drug Designation, re-enforcing the need for new and better treatments. With the Rare Pediatric Disease designation, Precision may be eligible to receive a Priority Review Voucher upon FDA approval of PBGENE-DMD.

In preclinical data presented at the ASGCT annual meeting in May 2025, PBGENE-DMD demonstrated significant and durable functional improvement in a humanized DMD mouse model. Following AAV delivery, PBGENE-DMD restored the body's ability to produce a functional dystrophin protein broadly across multiple muscle types, including cardiac and skeletal muscles. Over the course of nine months, mice treated with PBGENE-DMD showed increased dystrophin protein expression resulting in substantial and sustained functional muscle improvement. In addition, PBGENE-DMD-edited dystrophin mRNA transcript in muscle satellite stem cells, which are progenitor cells for new muscle cells, supports the potential for long-term durability.

In July 2025, Precision announced new preclinical data building upon previous data shared at the ASGCT annual meeting. These data demonstrated that PBGENE-DMD produced a three-fold increase in dystrophin-positive muscle cells between three and nine months in the quadricep, gastrocnemius (calf), heart, and diaphragm. In the gastrocnemius specifically, up to 85% of cells were dystrophin-positive, indicating a high degree of productive gene editing. This broad increase in dystrophin-positive cells combined with the increased dystrophin protein detected in tissues further validates the improved muscle function that was observed over time and may be attributable to edited satellite cells.

Precision is advancing the final U.S. investigational new drug (IND)-enabling toxicology studies with an anticipated IND and/or clinical trial application (CTA) filing targeted by the end of 2025 with initial 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. Precision has paused development of PBGENE-3243 to prioritize its two lead programs, PBGENE-HBV and PBGENE-DMD.

Partnered *In Vivo* Gene Editing 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. Preliminary data from the study presented at ASGCT in May demonstrated 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 of age and is eating appropriate levels of protein for a child of his age. iECURE has reported that a second infant with severe OTC deficiency was dosed in the first half of 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.

Non-Core *Ex Vivo* Programs

Azer-Cel (azercabtagene zapreleucel allogeneic CAR T treatment for cancer): Imugene Limited, Precision's clinical stage partner developing azer-cel for oncology indications, announced updated clinical data in July 2025. In the updated data in patients diagnosed with relapsed/refractory diffuse large B-cell lymphoma (DLBCL), two additional patients achieved a Complete Response and three additional patients achieved a Partial Response. Imugene reported that, as a result of the new data, the best overall response rate for azer-cel in DLBCL reached 75% and the Complete Response rate reached 55%. Based on the updated response rate and maturing durability data, as well as having been awarded FDA Fast Track Designation for DLBCL, Imugene expects to request an end of Phase 1 meeting with the FDA in the fourth quarter of 2025 to present the data and discuss designs for a pivotal/registrational trial for azer-cel.

Other Announcements

Mark Sulkowski, M.D., Professor of Medicine at the Johns Hopkins University School of Medicine and renowned expert in hepatic and infectious diseases has expanded his advisory role with Precision BioSciences. In the newly created role, Head Clinical Development Advisor, Dr. Sulkowski will work closely with Precision's leadership and cross-functional teams to support clinical strategy across the development lifecycle for the Company's on-going PBGENE-HBV Phase 1 clinical trial as well as initiation of later stage trials. His advisory role will focus on optimizing clinical trials, including translational integration, and aligning scientific rationale with regulatory objectives.

Quarter Ended June 30, 2025 Financial Results

“As Precision advances the ELIMINATE-B clinical trial and prepares to file an IND and/or CTA for the PBGENE-DMD program we have been closely managing our operating costs. Cost management is evident in our decision to pause development on PBGENE-3243 in the second quarter and is reflected in a $3.9 million reduction in our second quarter total operating expenses as compared to the same period last year,” said Alex Kelly, Chief Financial Officer of Precision BioSciences. “We have also extended our expected cash runway to the second half of 2027 to enable meaningful clinical data readouts for PBGENE-HBV and PBGENE-DMD. In July 2025, we initiated an operating efficiency program, including reductions in early research, manufacturing and general & administrative operating expenses which are aimed at reducing our annual cash operating expenses in each of 2026 and 2027 by approximately $25 million compared to the 2025 annual cash expense level.”

“In addition to significantly reducing our operating expenses, Precision will continue to pursue less dilutive sources of cash to even further extend the cash runway, including business development collaborations for future or deprioritized ARCUS programs as well as opportunities to monetize non-core program royalties and milestones,” added Mr. Kelly.

Cash, Cash Equivalents, and Restricted Cash: As of June 30, 2025, Precision had approximately $84.8 million in cash, cash equivalents, and restricted cash. Based on its expected cash runway, Precision believes it is sufficiently capitalized to reach important milestones for both programs, including commencement of a Phase 2 study for PBGENE-HBV and a potential pivotal trial for PBGENE-DMD. The Company expects existing cash and cash equivalents, upfront and potential near-term cash from CAR T transactions, along with expected operating efficiencies, operational receipts, and availability of Precision’s at-the-market (ATM) facility to extend Precision’s cash runway into the second half of 2027.

Revenues: Total revenues for the quarter ended June 30, 2025, were less than $0.1 million, as compared to $49.9 million for the quarter ended June 30, 2024. The decrease was expected and primarily the result of $48.2 million of non-cash revenue recognized in the prior period which represented all remaining deferred revenue related to the Prevail Therapeutics Agreement at its conclusion in April 2024 under generally accepted accounting principles. The upfront cash from this collaboration was received and recorded on the balance sheet in January 2021.

Research and Development Expenses: Research and development expenses were $12.8 million for the quarter ended June 30, 2025, as compared to $17.2 million for the quarter ended June 30, 2024. The decrease of $4.4 million was primarily due to decreases in PBGENE-HBV direct expenses due to lower manufacturing and toxicology expenses as the program transitioned to the clinic in the fall of 2024. PBGENE-3243, which has been paused, and other research related expenses also decreased in the comparative period, offset by an increase in PBGENE-DMD expenses as the program was accelerated in the second quarter of 2025.

General and Administrative Expenses: General and administrative expenses were $9.1 million for the quarter ended June 30, 2025, as compared to $8.5 million for the quarter ended June 30, 2024 as an increase in employee-related costs, including non-cash employee-related costs, offset a decrease in other general and administrative expenses.

Net Loss: Net loss was $23.5 million, or ($2.13) per share (basic and diluted), for the quarter ended June 30, 2025. Net income was $32.7 million, or $4.70 per share (basic) and $4.67 per share (diluted), for the quarter ended June 30, 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. 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 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, including timing of dose administrations and subsequent cohorts in the ELIMINATE-B trial; the unique design of PBGENE-HBV to eliminate cccDNA and inactivate integrated HBV DNA with high specificity, potentially leading to functional or complete cures; the suitability of PBGENE-HBV for the treatment of hepatitis and the targeting of the root cause of the disease; plans to escalate to higher dose levels and next cohorts in the ELIMINATE-B clinical trial; the potential of PBGENE-DMD to be a first-in-class *in vivo* gene editing approach addressing the majority of DMD patients; expectations on accelerated development of 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 expected timing and opportunities of regulatory processes (including IND-enabling studies and filings such as INDs or CTAs for PBGENE-HBV and PBGENE-DMD and the acceptance of these filings by regulatory agencies); the possibility and eligibility to receive a Priority Review Voucher upon FDA approval of PBGENE-DMD; 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, durability, and benefit of PBGENE-HBV and PBGENE-DMD, as well as our other product candidates and those being developed by partners; the complete enrollment of the OTC-HOPE study 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 2027 to advance PBGENE-HBV and PBGENE-DMD through meaningful Phase 1 data readouts and to enable commencement of a Phase 2 study for PBGENE-HBV and a potential pivotal trial for PBGENE-DMD; the potential of PBGENE-3243 as a first-of-its-kind treatment for m.3243-associated mitochondrial disease; the intention to pursue less dilutive sources of cash to even further extend the cash runway; and expectations from partners, including iECURE and Imugene Limited, on clinical data and timing and opportunities of regulatory processes. 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 June 30, 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 June 30,	
	2025	2024
Revenue	$ 18	$ 49,898
Operating expenses		
Research and development	12,768	17,225
General and administrative	9,127	8,527
Total operating expenses	21,895	25,752
Operating (loss) income	(21,877)	24,146
Other (expense) income:		
Loss from equity method investment	(665)	(950)
(Loss) gain on changes in fair value	(2,464)	694
Gain on change in fair value of warrant liability	753	7,765

Interest expense	(358)	(560)
Interest income	1,116	1,843
Loss on disposal of assets	(25)	(189)
Total other (expense) income	(1,643)	8,603
(Loss) income from operations	$ (23,520)	$ 32,749
Net (loss) income	$ (23,520)	$ 32,749
Net (loss) income per share		
Basic	$ (2.13)	$ 4.70
Diluted	$ (2.13)	$ 4.67
Weighted-average shares of common stock outstanding		
Basic	11,046,401	6,966,680
Diluted	11,046,401	7,011,630

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

	June 30, 2025	December 31, 2024
Cash, cash equivalents, and restricted cash	$ 84,806	$ 108,468
Working capital	56,691	80,009
Total assets	108,928	136,388
Total liabilities	74,874	79,995
Total stockholders' equity	$ 34,054	$ 56,393
Common stock outstanding	11,636,981	8,202,715

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