Precision BioSciences Reports Third Quarter 2024 Financial Results and Provides Business Update

November 4, 2024 at 7:01 AM EST

- Received approval for first Clinical Trial Application to advance PBGENE-HBV into first-in-human trials for treatment of chronic hepatitis B; additional global regulatory applications pending approval

- Opened PBGENE-HBV phase 1 clinical program in Moldova; patient screening underway with clinical data expected in 2025

- Strengthened infectious disease capabilities with key clinical talent added to senior leadership team

- Expected cash runway into the second half of 2026 with sufficient capital to phase 1 clinical data for multiple in vivo gene editing programs

- Company to host virtual investor event highlighting final PBGENE-HBV preclinical safety data and Phase 1 trial plans on November 15, 2024, at 7:00am PST / 10:00am EST

DURHAM, N.C.--(BUSINESS WIRE)--Nov. 4, 2024-- 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 sophisticated gene edits, today announced financial results for the third quarter ended September 30, 2024, and provided a business update.

“With the clearance of our first clinical trial application (CTA) for our lead program, PBGENE-HBV, we have arrived at the next phase of Precision’s journey as a single platform, clinical stage *in vivo* gene editing company. Our team is moving quickly to dose patients and in parallel is leveraging our robust regulatory package to seek additional regulatory application approvals globally with the aim of rapidly accelerating enrollment in the PBGENE-HBV phase 1 trial,” said Michael Amoroso, Chief Executive Officer at Precision BioSciences. “PBGENE-HBV represents the very first clinical stage gene editing program for chronic hepatitis B utilizing a differentiated dual modality targeting the elimination of cccDNA and inactivation of integrated HBV genomes - the root cause of viral persistence in chronic hepatitis B. We look forward to sharing detail on our clinical plans for PBGENE-HBV on November 15 prior to AASLD.”

“Looking ahead, we expect to report phase 1 PBGENE-HBV data throughout 2025 while continuing to work in parallel to submit an investigational new drug (IND) and/or CTA for our second wholly owned *in vivo* gene editing program, PBGENE-3243 for the potential treatment of m.3243-associated mitochondrial disease,” 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 potentially cure chronic hepatitis B. Currently, it is estimated that approximately 300 million people worldwide are afflicted with chronic hepatitis B. PBGENE-HBV is the first and only potentially curative gene editing program to enter clinic that is specifically designed to eliminate cccDNA and inactivate integrated HBV DNA.

In October 2024, Precision received CTA approval for PBGENE-HBV in Moldova and is working towards dosing patients. Investigators and clinical sites in Moldova, the first country to approve a CTA for PBGENE-HBV, have extensive experience executing and enrolling patients in early- and mid-stage hepatitis B clinical trials. Precision has submitted additional regulatory applications globally which are pending approval. The Company’s robust preclinical safety package supports the ability of PBGENE-HBV to specifically target and eliminate hepatitis B cccDNA and inactivate integrated HBV DNA without impacting any genes in the human genome, including no editing-associated translocations in HBV-infected primary human hepatocytes. In addition, non-human primate data showed that PBGENE-HBV was well-tolerated across multiple dose administrations.

PBGENE-3243 (Mutant Mitochondrial DNA Elimination Program previously known as PBGENE-PMM): PBGENE-3243 is a first-of-its-kind potential treatment for m.3243-associated mitochondrial diseases that is designed to target mutant mitochondrial DNA. Mitochondrial diseases are the most common hereditary metabolic disorder in the world. Precision has updated the program’s nomenclature to more accurately describe its intended target patient population – those who have m.3243 mutation and muscle-related symptoms. In particular, the m.3243-associated mitochondrial disease that PBGENE-3243 intends to address, affects approximately 20,000 people in the US alone. The high specificity of ARCUS nucleases enables editing and elimination of mutant mitochondrial DNA while allowing wild-type (normal) mitochondrial DNA to repopulate in the mitochondria, thus improving cellular function. Unlike CRISPR/Cas, base editors, and prime editors that require a guide RNA, ARCUS single-component nucleases do not require a nucleic acid for targeting and are able to penetrate the mitochondrial membranes.

Earlier this year, Precision presented additional data from the PBGENE-3243 program at the UMDF Conference. The presentation highlighted the ability of PBGENE-3243 to localize exclusively to mitochondria, avoiding any detectable off-target editing in the nuclear genome, while generating substantial shifts in heteroplasmy and improvements in mitochondrial function. The Company expects to submit an IND and/or CTA for this program in 2025.

Wholly Owned Portfolio – Under Assessment

In April 2024, Precision exercised its option to regain rights for the three programs developed under its collaboration with Prevail Therapeutics Inc. The Company is finalizing its portfolio assessment for these returned programs for internal development and/or development through new partners and

expects to provide an update as decisions are final. These programs include:

- **PBGENE-DMD** – novel gene excision approach for treatment of Duchenne Muscular Dystrophy utilizing a pair of ARCUS nucleases, delivered by a single adeno-associated virus (AAV), that are designed to *excise* an approximately 500,000 base pair mutation "hot spot" region from the dystrophin gene to generate a functionally competent variant of the dystrophin protein.
- **PBGENE-LIVER** – liver target for *gene insertion* with data demonstrating that ARCUS is capable of 40% to 45% high efficiency *gene insertion* at 1- and 3-months in nondividing cells, the most challenging context for *gene insertion*, in adult nonhuman primates.
- **PBGENE-CNS** – gene editing program targeting neurons to address a disease of the central nervous system.

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. iECURE expects initial data from this trial to be available in 2025.

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.

Corporate Updates & Upcoming Events

PBGENE-HBV Investor Event: Precision will host a virtual investor event on November 15, 2024, at 7:00am PST (10:00am EST) in San Diego highlighting the complete preclinical safety data and additional details regarding the phase 1 trial for PBGENE-HBV. The live webcast of the event will be available on the Events & Presentations section of the Precision BioSciences investor website.

ESGCT presentation: The company presented a poster at the European Society of Gene & Cell Therapy (ESGCT) 31st Annual Congress held on October 24, 2024, in Rome, Italy. The poster highlighted preclinical data demonstrating the ability of ARCUS to achieve high-efficiency *gene insertion, gene replacement*, and *base correction* via *homology-directed repair (HDR).* In the preclinical work presented, the company showed that targeted *gene insertion* can be achieved using ARCUS in greater than 85% of T cells and 39% of non-dividing primary human hepatocytes. These high rates of *gene insertion* were accomplished primarily through HDR which the research demonstrated was dependent on homology arms in the repair template and on the unique characteristic ARCUS 3' overhang cut in the direction of DNA replication.

Strengthened Senior Leadership Team: In September 2024, Precision announced the appointment of Dr. Murray Abramson, MD, MPH as Senior Vice President, Head of Clinical Development, and John Fry as Strategic Clinical Advisor, Hepatitis, significantly strengthening the Company's infectious disease and hepatitis capabilities to support its transition into the clinic and execute its Phase 1 PBGENE-HBV trial. Precision also announced the retirement of Alan List, MD who has assumed a role on Precision's Scientific Advisory Board as a Clinical Consultant.

Amended Banc of California Loan and Security Agreement: On July 31, 2024, the Company entered into an amended and restated loan and security agreement (the 2024 Loan and Security Agreement) with Banc of California (formerly known as Pacific Western Bank) pursuant to which Banc of California provided the Company with a term loan with a principal amount of $22.5 million secured by restricted cash. The maturity date under the 2024 Loan and Security Agreement is June 30, 2027.

Quarter Ended September 30, 2024 Financial Results:

Cash, Cash Equivalents, and Restricted Cash: As of September 30, 2024, Precision had approximately $121.3 million in cash, cash equivalents, and restricted cash. The Company expects that existing cash and cash equivalents, upfront and 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 are expected 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 propel two wholly owned programs through Phase 1 data readouts in 2025 and 2026.

Revenues: Total revenues for the quarter ended September 30, 2024, were $0.6 million, as compared to $13.1 million for the same period in 2023. The decrease of $12.5 million in revenue during the quarter ended September 30, 2024, was primarily the result of a $7.0 million decrease in revenue recognized under the Novartis Agreement as Precision nears completion of its pre-clinical workplan compared to the three months ended September 30, 2023. In addition, there was a $5.5 million decrease in revenue recognized under the Prevail Agreement during the three months ended September 30, 2024, following conclusion of the collaboration in April 2024.

Research and Development Expenses: Research and development expenses were $13.1 million for the quarter ended September 30, 2024, as compared to $15.9 million for the same period in 2023. The decrease of $2.8 million was primarily due to a $2.7 million decrease in PBGENE-HBV external development costs primarily from nonclinical studies and a $2.0 million decrease in outsourced R&D costs, lab supplies and services, and share based compensation, offset by a $2.4 million increase in PBGENE-3243 program external development costs as the program continues to advance toward an IND and/or CTA in 2025.

General and Administrative Expenses: General and administrative expenses were $8.8 million for the quarter ended September 30, 2024, as compared to $9.6 million for the same period in 2023. The decrease of $0.8 million was primarily due to a decrease in consulting fees in addition to decreases in tax and insurance expenses.

Net Loss from Continuing Operations: Net loss from continuing operations was $16.4 million for the quarter ended September 30, 2024, as compared to a net loss from continuing operations of $12.1 million for the same period in 2023. The increase in net loss was primarily related to decreases in revenue under the Novartis and Prevail Agreements.

Net Loss: Net loss was $16.4 million, or $(2.25) per share (basic and diluted), for the quarter ended September 30, 2024, as compared to a net loss of

$8.1 million, or $(2.10) per share (basic and diluted), for the same period in 2023.

Shares: Basic and diluted weighted-average common shares outstanding for the third quarter of 2024 were 7,287,173 compared to 3,838,900 for the same period in 2023. Precision BioSciences had 7,480,521 shares outstanding as of September 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).

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 and our partners' and licensees' product candidates and 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 (including filings, studies, enrollment and clinical data for PBGENE-HBV, PBGENE-PMM and iECURE OTC); the design of PBGENE-HBV to directly eliminate cccDNA and inactivate integrated HBV DNA with high specificity, potentially leading to functional cures; the ability of ARCUS single-component nucleases to penetrate the mitochondrial membranes; expectations about our and our partners' operational initiatives, strategies, and further development of our programs; expectations and updates around our partnerships and collaborations and our ability to enter into new collaborations, license agreements or other arrangements; our expected cash runway and available credit; the sufficiency of our cash runway extending into the second half of 2026 and realizing Phase 1 clinical data for multiple *in vivo* gene editing programs; expectations about achievement of key milestones and receipt of any milestone, royalty, or other payments; expectations regarding our liquidity and capital resources; and anticipated timing of clinical data . In some cases, you can identify forward-looking statements by terms such as "aim," "anticipate," "approach," "believe," "contemplate," "could," "designed," "estimate," "expect," "goal," "intend," "look," "may," "mission," "plan," "possible," "potential," "predict," "project," "pursue," "should," "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.

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

	For the Three Months Ended September 30,	
	2024	**2023**
Revenue	$ 576	$ 13,120
Operating expenses		
Research and development	13,084	15,850
General and administrative	8,767	9,633
Total operating expenses	21,851	25,483
Operating loss	(21,275)	(12,363)
Other income (expense), net:		
Loss from equity method investment	(875)	(1,350)
Gain on change in fair value	571	311
Gain on change in fair value of warrant liability	3,647	—
Interest expense	(256)	(576)
Interest income	1,763	1,870
Loss on disposal of assets	—	(2)
Total other income	4,850	253
Loss from continuing operations	$ (16,425)	$ (12,110)
Income from discontinued operations	—	$ 4,031
Net loss	$ (16,425)	$ (8,079)
Net loss per share		
Basic	$ (2.25)	$ (2.10)
Diluted	$ (2.25)	$ (2.10)
Weighted-average shares of common stock outstanding		
Basic	7,287,173	3,838,900
Diluted	7,287,173	3,838,900

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

(Unaudited)

	September 30, 2024	December 31, 2023
Cash, cash equivalents, restricted cash	$ 121,328	$ 116,678
Working capital	98,541	86,372
Total assets	153,258	159,781
Total liabilities	88,392	140,920
Total stockholders' equity	64,866	18,861
Common stock outstanding	7,480,521	4,164,038

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