# Precision BioSciences Reports Third Quarter 2023 Financial Results and Provides Business Update

November 7, 2023 at 7:00 AM EST

*- Precision to present late breaker PBGENE-HBV data at The Liver Meeting of AASLD in November 2023*

*- Hosted R&D Day highlighting exclusive focus on in vivo gene editing pipeline*

*- Expanded portfolio with PBGENE-PMM as a potentially curative treatment for m.3243 associated primary mitochondrial myopathy*

*- Completed strategic transaction with Imugene for azer-cel in cancer*

*- Extended cash runway through the end of 2025*

DURHAM, N.C.--(BUSINESS WIRE)--Nov. 7, 2023-- Precision BioSciences, Inc. (Nasdaq: DTIL), an advanced gene editing company utilizing its novel proprietary ARCUS® platform to develop *in vivo* gene editing therapies for sophisticated gene edits, including gene insertion, excision, and elimination, today announced financial results for the third quarter ended September 30, 2023 and provided a business update.

"The ARCUS platform has always been foundational to Precision, as we have harnessed its distinct properties in pursuit of life changing medicines for patients. Our recent transaction with Imugene allows Precision to focus the power of ARCUS on *in vivo* gene editing while capturing near-term and potential long-term value for our CAR T assets," said Michael Amoroso, Chief Executive Officer at Precision BioSciences. "At our recent R&D Day, we featured data demonstrating how the differentiated cut, size, and simplicity of ARCUS enable it to uniquely carry out sophisticated edits that present clear advantages in the market and compelling therapeutic opportunities versus other editing technologies. By design, ARCUS has been shown to preferentially drive higher efficiency gene insertion than CRISPR-derived editors, and its ease of delivery and simplicity confer greater utility in reaching tissues, organs, and organelles for gene insertion, excision and elimination. Going forward, Precision will solely focus its own efforts on programs leveraging ARCUS' specific utility for sophisticated edits. To that end, we are progressing wholly-owned PBGENE-HBV and PBGENE-PMM programs towards anticipated CTA and/or IND filings in 2024 and 2025, respectively. In addition, the ARCUS OTC gene insertion program being developed by our partner at iECURE is targeting a CTA and/or IND filing by the end of this year. Importantly, as we execute against our singular focused *in vivo* strategy, we believe we will have sufficient capital to advance these wholly-owned programs into the clinic and to Phase 1 data points over the next two years."

**Completed Strategic Transaction with Imugene for Azer-Cel in Cancer**

In August 2023, the Company completed a strategic transaction with Imugene Limited (ASX: IMU) for the Company's lead allogeneic chimeric antigen receptor (CAR) T candidate for cancer, azercabtagene zapreleucel (azer-cel). In exchange for global rights to azer-cel for cancer, as well as Precision's CAR T infrastructure and experienced cell therapy teams, Precision received upfront cash and equity consideration valued at $21 million. In addition, Precision is eligible for a potential $8 million near-term milestone payment, up to $198 million in additional milestone payments and double-digit royalties on net sales of azer-cel, as well as $145 million in milestone payments and tiered royalties for up to three additional research programs to be potentially developed by Imugene. Imugene has assumed ongoing clinical execution for azer-cel in the large B-cell lymphoma population who have relapsed following autologous CAR T treatment.

**Advancing as a Single Platform Company Focused on ARCUS *In Vivo* Gene Editing**

Precision is now solely focused on leveraging its proprietary ARCUS genome editing platform to advance *in vivo* gene editing programs that go beyond gene knockouts in the liver and carry out more sophisticated edits such as gene insertions, gene excision, and gene elimination unlocking a broader potential for ARCUS *in vivo* gene editing in human therapeutics.

In support of the go-forward strategy, Precision presented two poster presentations at the European Society of Gene & Cell Therapy congress on October 25 and 26, 2023, in Brussels, Belgium, "*Unique features of ARCUS nucleases enable high efficiency, targeted gene insertion in vivo"* and "*ARCUS-mediated excision of the "hot spot" region of the human dystrophin gene results in functional improvement in a mouse model of Duchenne muscular dystrophy (DMD)*."

**Wholly-owned Portfolio**

**PBGENE-HBV (Viral Elimination Program):** Precision is developing PBGENE-HBV for the treatment of patients with chronic hepatitis B and expects to submit a clinical trial application (CTA) and/or investigational new drug (IND) application in 2024.

Chronic infection with hepatitis B virus (HBV) is due to persistence of the viral genome, in the form of covalently closed circular DNA (cccDNA) and viral sequences integrated into the human genome. Current treatments for chronic hepatitis B rarely achieve a functional cure, defined as sustained undetectable levels of circulating hepatitis B surface antigen (HBsAg) and HBV DNA after a finite course of treatment, as they do not eliminate the root cause of viral persistence. In September 2023, at its R&D Day, the Company shared data highlighting the potential of PBGENE-HBV to drive sustained reductions of circulating HBsAg and HBV DNA. Unlike other therapies in development, PBGENE-HBV directly eliminates cccDNA and inactivates integrated viral sequences, representing a potentially curative approach.

A late-breaking abstract featuring preclinical data on the PGBENE-HBV program has been accepted for a poster presentation, "*Preclinical efficacy and*

*safety of ARCUS-POL nucleases for chronic hepatitis B: a potentially curative strategy,*" at the American Association for the Study of Liver Diseases (AASLD) Annual Meeting being held in Boston on November 10-14, 2023.

**PBGENE-PMM (Mutant Mitochondrial Elimination Program):** At its R&D Day, Precision announced that it will pursue development of PBGENE-PMM as a potential first-in-class opportunity for treatment of m.3243-associated primary mitochondrial myopathy (PMM). Mitochondrial diseases are the most common hereditary metabolic disorder, affecting 1 in 4,300 people. PMM currently lacks a curative treatment and impacts approximately 50% of patients with mitochondrial disease. The high specificity and simplistic, single component nature of Precision's mitoARCUS nucleases are designed to enable specific editing to eliminate mutant mitochondrial DNA while allowing normal (wild-type) mitochondrial DNA to repopulate in the mitochondria and restore normal function. Precision is targeting to submit a CTA and/or IND in 2025 for PBGENE-PMM.

In September 2023, Precision received a Notice of Allowance from the U.S. Patent and Trademark Office (USPTO) for U.S. Patent Application No. 18/161,560, titled "Engineered Meganucleases That Target Human Mitochondrial Genomes." The allowed composition of matter claims encompass a mitochondria-targeted ARCUS nuclease (mitoARCUS) that is designed to specifically target, cleave, and eliminate mutant mitochondrial DNA comprising an m.3243A>G mutation.

**Partnered Programs**

**iECURE-OTC (Gene Insertion Program):** Led by iECURE, an ARCUS-mediated gene insertion approach is being pursued as a potential treatment for neonatal onset ornithine transcarbamylase (OTC) deficiency. Non-human primate (NHP) data presented in October 2022 by researchers from the University of Pennsylvania's Gene Therapy Program demonstrated sustained gene insertion of a therapeutic OTC transgene one-year post-dosing in newborn and infant NHPs with high efficiency. iECURE expects to submit a CTA and/or IND in the second half of 2023.

**PBGENE-NVS (Gene Insertion Program):** Precision continues to advance its *in vivo* 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 into hematopoietic stem cells as a potential one-time transformative treatment administered directly to the patient that would overcome many of the hurdles present today with other therapeutic technologies, including *ex vivo* gene editing approaches.

**PBGENE-DMD (Gene Excision Program):** Precision continues its *in vivo* gene editing collaboration with Prevail Therapeutics, a wholly-owned subsidiary of Eli Lilly and Company, in applying ARCUS nucleases to three initial targets, including DMD in muscle, a liver-directed target, and a central nervous system-directed target. The goal of the PBGENE-DMD program is to utilize a pair of ARCUS nucleases, delivered by a single AAV, that are designed to excise an approximately 500,000 base pair mutation "hot spot" region from the dystrophin gene to generate a variant of the dystrophin protein that is functionally competent. During its September 2023 R&D Day, the Company highlighted preclinical data demonstrating the potential of ARCUS *in vivo* gene editing for large gene excisions and that the edited dystrophin variant was observed in multiple tissue types frequently involved in progression of DMD, including skeletal muscle, heart, and diaphragm, thereby enabling significantly improved muscle function.

**Quarter Ended September 30, 2023 Financial Results:**

In August 2023, the Company announced its final stages of execution to operate as a single platform company focused exclusively on developing *in vivo* gene editing therapies with the completion of the strategic transaction with Imugene for azer-cel for cancer. Precision determined that the decision to only pursue development of CAR T cell therapies through partnerships qualified for discontinued operations accounting treatment. Accordingly, the accompanying financial statements for all periods presented reflect Precision's CAR T cell therapy business as a discontinued operation.

**Cash and Cash Equivalents:** As of September 30, 2023, Precision had approximately $122.2 million in cash and cash equivalents. The Company expects that existing cash and cash equivalents, expected operational receipts, including upfront consideration received from Imugene, operational efficiencies gained from divestment of the CAR T business, availability of the ATM facility, and available credit will be sufficient to fund its operating expenses and capital expenditure requirements through the end of 2025. The Company expects its cash runway to be sufficient to achieve first-in-human Phase 1 clinical data for its lead *in vivo* gene editing programs.

**Revenues:** Total revenues for the quarter ended September 30, 2023 were $13.1 million, as compared to $7.4 million for the same period in 2022. The increase of $5.7 million in revenue during the quarter ended September 30, 2023 was primarily the result of an increase of $4.0 million in revenue recognized under the Novartis Agreement and an increase of $1.7 million in revenue recognized under the Prevail Agreement.

**Research and Development Expenses:** Research and development expenses were $15.9 million for the quarter ended September 30, 2023, as compared to $11.8 million for the same period in 2022. The increase of $4.1 million was primarily due to $5.1 million in expenses related to initiating CTA/IND-enabling studies for the PBGENE-HBV development program planned for CTA/IND submission in 2024, offset by decreases in laboratory supplies and services and share-based compensation expense. R&D expenses in the quarter ended September 30, 2023 are not necessarily indicative of future spend as work may fluctuate quarter to quarter in the pre-CTA/IND phase.

**General and Administrative Expenses:** General and administrative expenses were $9.6 million for the quarter ended September 30, 2023, as compared to $10.3 million for the same period in 2022. The decrease of $0.7 million was primarily due to a decrease in share-based compensation expense offset by an increase in information technology expenses.

**Interest Income/Expense:** Interest expense was $0.6 million for the quarter ended September 30, 2023 compared to $0.4 million for the same period in 2022, primarily due to higher interest rates on our debt.

Interest income was $1.9 million during the quarter ended September 30, 2023 compared to $1.2 million for the same period in 2022. The increase in interest income was primarily the result of higher interest rates on our cash and cash equivalents compared to the three months ended September 30, 2022.

**Discontinued Operations:** Income from discontinued operations was $4.0 million during the three months ended September 30, 2023 compared to an $8.3 million loss during the three months ended September 30, 2022. The $12.3 million increase was the result of the $8.4 million gain on sale of our CAR T infrastructure to Imugene and a $3.9 million decrease in cell therapy expenses during the three months ended September 30, 2023 as compared to the three months ended September 30, 2022.

**Net Loss:** Net loss was $8.1 million, or $(0.07) per share (basic and diluted), including $4.0 million income from discontinued operations for the quarter ended September 30, 2023. Net loss was $23.9 million, or $(0.22) per share (basic and diluted), including a $8.3 million loss from discontinued

operations for the same period in 2022.

**About Precision BioSciences, Inc.**

Precision BioSciences, Inc. is an advanced 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.

**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 and gene editing approaches including ARCUS's potential editing efficiency and differentiating aspects and the ability for the ARCUS genome editing platform to develop differentiated programs; the suitability of ARCUS nucleases for gene or viral elimination, large excision, gene insertion, and other complex gene editing approaches to drive defined outcomes; the potential of PBGENE-HBV to drive sustained reductions of circulating HBsAg and HBV DNA; the expected timing of regulatory processes, including timing of expected CTA and/or IND filings; expectations about our operational initiatives and business strategy; expectations around the Company's partnerships; expectations about achievement of key milestones; and expectations regarding the Company's cash runway, including the assumptions underlying the Company's calculations of its cash runway and whether its cash runway will be sufficient to provide first-in-human clinical data for the Company's research and development programs. The words "aim," "anticipate," "approach," "believe," "contemplate," "could," "designed", "estimate," "expect," "explore," "goal," "intend," "look," "may," "mission," "plan," "possible," "potential," "predict," "project," "promise," "pursue," "seek" "should," "target," "will," "would," and other similar words or expressions, or the negative of these words or similar words or expressions, are intended to identify forward-looking statements, though not all forward-looking statements use these words or 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 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' ability to identify, develop and commercialize product candidates; pending and 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' ability to advance product candidates into, and successfully design, implement and complete, clinical or field trials; 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, 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, 2023, as any such factors may be updated from time to time in our other filings with the Securities and Exchange Commission ("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, | |
|---|---|---|
| | **2023** | **2022** |

| | | |
|---|---|---|
| Revenue | $ 13,120 | $ 7,363 |
| Operating expenses | | |
| Research and development | 15,850 | 11,758 |
| General and administrative | 9,633 | 10,281 |
| Total operating expenses | 25,483 | 22,039 |
| Operating loss | (12,363) | (14,676) |
| Other income (expense): | | |
| Loss from equity method investment | (1,350) | (1,782) |
| Gain on changes in fair value | 311 | — |
| Interest expense | (576) | (405) |
| Interest income | 1,870 | 1,172 |
| Loss on disposal of assets | (2) | — |
| Total other income (expense) | 253 | (1,015) |
| Loss from continuing operations | $ (12,110) | $ (15,691) |
| Income (loss) from discontinued operations (including gain on disposal of $8,446 during the three and nine months ended September 30, 2023) | 4,031 | (8,255) |
| Net Loss | $ (8,079) | $ (23,946) |
| Net loss per share attributable to common stockholders-basic and diluted | $ (0.07) | $ (0.22) |
| Weighted average shares of common stock outstanding-basic and diluted | 115,174,752 | 110,849,196 |

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

| | September 30, 2023 | December 31, 2022 |
|---|---|---|
| Cash and cash equivalents | $ 122,231 | $ 189,576 |
| Working capital | 95,235 | 139,441 |
| Total assets | 164,344 | 238,169 |
| Total liabilities | 135,281 | 177,736 |
| Total stockholders' equity | $ 29,063 | $ 60,433 |
| Common stock outstanding | 115,680,008 | 110,964,035 |

View source version on businesswire.com: https://www.businesswire.com/news/home/20231107837870/en/

**Investor and Media Contact:**
Mei Burris
Senior Director of Finance and Corporate Controller
Mei.Burris@precisionbiosciences.com

Source: Precision BioSciences, Inc.