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

August 1, 2024 at 7:15 AM EDT

- Wholly owned programs PBGENE-HBV for Chronic Hepatitis B virus and PBGENE-PMM for m.3243 mitochondrial disease on track for IND and/or CTA submissions in 2024 and 2025, respectively

- Expanded Hepatitis Scientific Advisory Board with addition of world-class clinical investigators Mark Sulkowski, M.D. and Jordan Feld, M.D., M.P.H.

- Sufficient capital to realize Phase 1 clinical data for multiple in vivo gene editing programs; Expected cash runway into the second half of 2026

- Regained control of three advanced preclinical programs for development internally or with partners, including a novel gene editing approach for Duchenne Muscular Dystrophy

DURHAM, N.C.--(BUSINESS WIRE)--Aug. 1, 2024-- 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 elimination, gene insertion, and gene excision, today announced financial results for the second quarter ended June 30, 2024 and provided a business update.

“During the first half of 2024 we have made excellent progress against our key priorities. We rapidly advanced PBGENE-HBV toward planned investigational new drug (IND) submission and/or clinical trial application (CTA) in 2024. We believe we are in a strong position to advance our first wholly owned *in vivo* gene editing program into the clinic. PBGENE-HBV is expected to be the first and only potentially curative gene editing program to enter the clinic that is specifically designed to provide a functional cure for chronic Hepatitis B by eliminating the root source of viral replication. I’m proud of our team’s operational progress this year, including nearing completion of final toxicology studies, production of final clinical trial material, onboarding global clinical study sites in multiple countries to conduct the Phase 1 trial, and adding world-class clinicians to our Hepatitis Scientific Advisory Board,” said Michael Amoroso, President and Chief Executive Officer of Precision BioSciences.

“While advancing PBGENE-HBV as our first operational priority in 2024, we have also made steady progress on our second wholly owned program PBGENE-PMM for 3243 mutated (m.3243) mitochondrial disease, which is targeted for IND and/or CTA filing in 2025.”

“As we enter the second half of the year, we are sufficiently capitalized based on our expected cash runway as a result of multiple business development deals combined with a $40 million equity raise to propel our two wholly owned programs to Phase 1 data readouts in 2025 and 2026,” emphasized Mr. Amoroso.

Wholly Owned Portfolio

PBGENE-HBV (Viral Elimination Program): Precision is developing PBGENE-HBV for the treatment of patients with chronic Hepatitis B. Currently, it is estimated that approximately 300 million people worldwide are afflicted with chronic Hepatitis B. PBGENE-HBV is expected to be 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.

In June 2024, Precision participated in a panel discussion on the application of gene editing to the treatment of chronic Hepatitis B during the HBV Forum and presented new preclinical safety data for PBGENE-HBV at the European Association for the Study of the Liver. This data further supported the ability of PBGENE-HBV to specifically target and cut HBV DNA, leading to the elimination of cccDNA and integrated HBV DNA. The data also demonstrated a lack of detectable off-target editing for PBGENE-HBV at therapeutically relevant doses, including no editing-associated translocations in HBV infected primary human hepatocytes. Importantly, new non-human primate data showed that PBGENE-HBV was well-tolerated across multiple dose administrations. PBGENE-HBV is advancing through final toxicology studies and drug for the planned Phase 1 clinical trial has been manufactured. Precision expects to submit an IND and/or CTA for this program in 2024 and plans to provide an additional program update later this year.

PBGENE-PMM (Mutant Mitochondrial DNA Elimination Program): PBGENE-PMM is a first of its kind potential treatment for m.3243-mitochondrial disease designed to target mutant mitochondrial DNA while having no adverse impact on wild type (normal) mitochondrial DNA. Mitochondrial diseases are the most common hereditary metabolic disorder in the world. The m.3243 mitochondrial disease population that the program intends to address is large, affecting approximately 20,000 people in the US alone. The highly specific ARCUS nuclease is designed to shift heteroplasmy by editing and eliminating mutant mitochondrial DNA while allowing wild type mitochondrial DNA to repopulate in the mitochondria, thus improving cellular function. Unlike CRISPR/Cas, base editors, and prime editors, ARCUS single-component nucleases do not require a guide RNA and are therefore differentiated amongst gene editing modalities due to their ability to penetrate mitochondrial membranes.

In June 2024, Precision presented additional data from the PBGENE-PMM program at the United Mitochondrial Disease Foundation Mitochondrial Medicine 2024 Conference. The presentation highlighted the ability of PBGENE-PMM 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 anticipates filing an IND and/or CTA for this program in 2025.

Wholly Owned Portfolio – Under Assessment

In July 2024, Precision regained control of three programs developed under its collaboration with Prevail Therapeutics Inc. The Company has received inbound interest from potential partners regarding these programs and is in the process of conducting a portfolio assessment for these returned programs for internal development and/or development through new partners and expects to provide an update as decisions are finalized. 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 restore a functionally competent variant of the native dystrophin protein. We believe this approach is unique when compared with microdystrophin treatment and is the first in class gene editing application for Duchenne Muscular Dystrophy.
- **PBGENE-LIVER** – liver target for gene insertion with data demonstrating that ARCUS achieved 40% to 45% high efficiency and durable gene insertion at 1- and 3-months in nondividing cells in adult nonhuman primates (NHPs), the most challenging context for gene insertion. To our knowledge, ARCUS is the only gene editor presented at a conference showing high efficiency gene insertion in non-dividing cells in NHPs. This opens therapeutic application for both pediatric patients whose cells divide quickly, as well as adult patients whose cells divide much less frequently than pediatric patients.
- **PBGENE-CNS** – gene editing program targeting neurons to address a disease of the central nervous system. Precision is the first to demonstrate successful *in vivo* editing of neurons in both mice and NHPs. This remains a very attractive program for Precision or for partners focused on neurodegenerative diseases.

Partnered Programs:

iECURE-OTC (Gene Insertion Program for OTC deficiency): Led by iECURE, ECUR-506 is the first ARCUS-mediated *in vivo* gene editing program to advance into the clinic following regulatory approvals in the US, the United Kingdom, and Australia for initiation of the OTC-HOPE study. The OTC-HOPE study is a first-in-human Phase 1/2 trial evaluating ECUR-506 as a potential treatment for neonatal onset ornithine transcarbamylase (OTC) deficiency and has begun recruiting patients at two sites in the United Kingdom. In May 2024, iECURE announced that it had received Fast Track designation from the FDA for ECUR-506 and expects initial data from this trial to be available in late 2024 or in 2025.

PBGENE-NVS (Gene Insertion Program for Sickle Cell Anemia and Beta Thalassemia): 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. We believe this is the only *in vivo* approach in development that can be administered directly to the patient to overcome disparities in patient treatment access with other therapeutic technologies, including those that are targeting an *ex vivo* gene editing approach.

Corporate Updates

Expansion of Hepatitis Scientific Advisory Board to include world-class clinical investigators: In June 2024, Precision announced the appointment of Mark Sulkowski, M.D. and Jordan Feld, M.D., M.P.H. to its Hepatitis Scientific Advisory Board. They join inaugural member Raymond Schinazi, Ph.D., DSc, to provide counsel and deepen the Company’s scientific and clinical expertise ahead of Precision’s anticipated IND and/or CTA submission for PBGENE-HBV.

Addition to the Russell Microcap® Index: As of the close of U.S. markets on June 28, 2024, Precision BioSciences was added to the Russell Microcap Index as part of the index’s annual reconstitution.

Common Stock purchase by members of management for $300,000: In May 2024, Precision entered into a definitive subscription agreement, pursuant to which the Company issued and sold in a non-brokered private placement to members of its senior leadership team, including the Chief Executive Officer, 25,000 shares of its common stock at a price of $12.00 per share, representing a 13.5% premium to the closing price of its common stock immediately preceding the signing of the subscription agreement, for an aggregate amount of $300,000.

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. The proceeds from the term loan were used to repay the $22.5 million outstanding principal balance under the prior revolving line of credit with Banc of California, and pursuant to the terms of the 2024 Loan and Security Agreement, the revolving line of credit was terminated. The maturity date under the 2024 Loan and Security Agreement is June 30, 2027. The term loan bears interest at an annual rate equal to the greater of (i) 1.50% below the Prime Rate then in effect or (ii) 4.50%.

Quarter Ended June 30, 2024 Financial Results:

Cash and Cash Equivalents: As of June 30, 2024, Precision had approximately $123.6 million in cash and cash equivalents. 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, availability of Precision’s at-the-market (ATM) facility, and available credit are expected to extend Precision’s cash runway into the second half of 2026.

Revenues: Total revenues for the quarter ended June 30, 2024 were $49.9 million, as compared to $19.8 million for the same period in 2023. The increase of $30.1 million in revenue during the quarter ended June 30, 2024 was primarily the result of recognizing deferred revenue related to the termination of the amended and restated development and license agreement with Prevail Therapeutics.

Research and Development Expenses: Research and development expenses were $17.2 million for the quarter ended June 30, 2024, as compared to $13.1 million for the same period in 2023. The increase of $4.1 million was primarily due to an increase in external development costs for the PBGENE-HBV and PBGENE-PMM programs as they continue to advance towards the clinic.

General and Administrative Expenses: General and administrative expenses were $8.5 million for the quarter ended June 30, 2024, as compared to

$9.8 million for the same period in 2023. The decrease of $1.3 million was primarily due to decreased employee and share-based compensation expense from a decrease in headcount.

Net Income from Continuing Operations: Net income from continuing operations was $32.7 million for the quarter ended June 30, 2024, inclusive of a $7.8 million non-cash gain on the fair value of our warrant liability which does not impact our cash runway, as compared to a net loss from continuing operations of $11.9 million, for the same period in 2023. The improvement was primarily related to recognition of deferred revenue from termination of the Prevail Agreement as well as the non-cash gain related to the fair value adjustments of our warrant liability.

Net Income: 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, as compared to a net loss of $11.9 million, or $(3.13) per share (basic and diluted), for the same period in 2023.

Shares: Basic and diluted weighted-average common shares outstanding for the second quarter of 2024 were 6,966,680 and 7,011,630, respectively, compared to 3,803,083 (basic and diluted) for the same period in 2023.

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.

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 (including filings and studies for PBGENE-HBV and PBGENE-PMM); 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; 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 June 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 June 30,	
	2024	**2023**
Revenue	$ 49,898	$ 19,789
Operating expenses		
Research and development	17,225	13,088
General and administrative	8,527	9,830
Total operating expenses	25,752	22,918
Operating income (loss)	24,146	(3,129)
Other income (expense):		
Loss from equity method investment	(950)	(1,369)
Gain on changes in fair value	694	—
Gain on change in fair value of warrant liability	7,765	—
Interest expense	(560)	(553)
Interest income	1,843	1,946
(Loss) gain on disposal of assets	(189)	72
Total other income	8,603	96
Income (loss) from continuing operations	$ 32,749	$ (3,033)
Loss from discounted operations	—	(8,858)
Net income (loss)	$ 32,749	$ (11,891)
Net income (loss) per share		
Basic	$ 4.70	$ (3.13)
Diluted	$ 4.67	$ (3.13)
Weighted-average shares of common stock outstanding		
Basic	6,966,680	3,803,083
Diluted	7,011,630	3,803,083

Precision Biosciences, Inc.

Condensed Balance Sheets Data
(In thousands, except share amounts)
(Unaudited)

	June 30, 2024	December 31, 2023
Cash and cash equivalents	$ 123,571	$ 116,678
Working capital	111,078	86,372
Total assets	165,800	159,781
Total liabilities	91,086	140,920
Total stockholders' equity	$ 74,714	$ 18,861
Common stock outstanding	7,123,370	4,164,038

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Source: Precision BioSciences, Inc.