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

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- Wholly owned programs on track for CTA and/or IND submissions - PBGENE-HBV for Hepatitis B virus in 2024 and PBGENE-PMM for primary mitochondrial myopathy in 2025

- First ARCUS in vivo gene editing program now open for enrollment in the United Kingdom through partnered OTC deficiency program; United States and Australia expected to follow

- Exercised option to return three advanced preclinical programs for development internally or with partners, including a novel gene editing approach for Duchenne Muscular Dystrophy

- Monetized CAR T assets through licensing deals with TG Therapeutics and Caribou Biosciences, and completed $40 million common stock offering extending expected runway into H2 2026

DURHAM, N.C.--(BUSINESS WIRE)--May 13, 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, insertion, and excision, announced financial results for the first quarter ended March 31, 2024, and provided a business update.

“In 2024, Precision is focused on strong execution as we advance our wholly owned Hepatitis B virus (HBV) and primary mitochondrial myopathy (PMM) *in vivo* gene editing programs towards the clinic as soon as possible,” said Michael Amoroso, Chief Executive Officer at Precision BioSciences. “In parallel, our partner, iECURE has already advanced the first ARCUS *in vivo* gene editing program for OTC deficiency into the clinic. We believe this not only validates ARCUS novel editing approach for gene insertion, but also sets key regulatory precedence for *in vivo* ARCUS gene editing programs across global markets including the US.”

“We were pleased to regain control of three exciting development opportunities from our work with Prevail Therapeutics, which take advantage of ARCUS’ unique cut, size, and simplicity. Strong *in vivo* proof-of-concept data generated to date have yielded three advanced preclinical programs that we believe have first-in-class and best-in-class potential across multiple therapeutic areas. We are currently assessing which programs Precision will advance internally and seek to re-partner and expect to provide an update once plans have been finalized,” added Mr. Amoroso.

“In addition to the important operational progress we continue to make with our wholly owned and partnered programs, we added cash to our balance sheet by monetizing our CAR T assets and completing a $40 million equity offering to extend our cash runway into the second half of 2026 to fund continued development of our programs.”

Wholly Owned Portfolio – Lead Programs

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 February 2024, Precision announced that the company had received pre-IND regulatory feedback from the US Food and Drug Administration (FDA) in addition to regulatory feedback from agencies outside the US providing clarity and alignment on PBGENE-HBV investigational new drug (IND)/clinical trial application (CTA)-enabling preclinical plans and clinical strategy. PBGENE-HBV is advancing through final toxicology studies and Precision expects to submit an IND and/or CTA for this program in 2024.

PBGENE-PMM (Mutant Mitochondrial DNA Elimination Program): PBGENE-PMM is a first of its kind potential treatment for m.3243-associated PMM by targeting mutant mitochondrial DNA. Mitochondrial diseases are the most common hereditary metabolic disorder in the world. The m.3243 associated PMM that our program intends to address is sizable, affecting up to 25,000 people in the US alone. Precision scientists published new preclinical data in *Nature Metabolism* highlighting the high specificity of ARCUS nucleases designed to edit and eliminate mutant mitochondrial DNA while allowing wild-type (normal) mitochondrial DNA to repopulate in the mitochondria, thus improving cellular function. This is a differentiated program because unlike CRISPR/Cas, base editors, and prime editors, ARCUS nucleases are able to penetrate the mitochondrial membranes because they are single-component editors that do not require a guide RNA. The Company expects to submit an IND and/or CTA for PBGENE-PMM in 2025 for this program.

Wholly Owned Portfolio – Under Assessment

As previously announced, Precision’s collaboration with Prevail Therapeutics Inc., has concluded, and Precision exercised its option to regain rights for the three programs developed under the collaboration. The Company is in the process of conducting a portfolio assessment for the newly 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 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 – *In Vivo* Gene Editing

iECURE-OTC (Gene Insertion Program): 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 screening patients. iECURE has recently communicated that one site in the United Kingdom is open and recruiting patients for the OTC-HOPE study. Non-human primate (NHP) data presented 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 NHP’s with high efficiency. iECURE has 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): 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.

Business Updates – Monetization of CAR T Investments:

Completed Licensing Deal with TG Therapeutics for Cell Therapy Azer-Cel in Autoimmune Diseases, and Other Indications Outside of Cancer:

In January 2024, Precision announced the completion of a transaction with TG Therapeutics (Nasdaq: TGTX) for certain exclusive and non-exclusive license rights to develop Azercabtagene Zapreleucel (azer-cel) for autoimmune diseases and other indications outside of cancer. In exchange for these rights, Precision received upfront and potential near-term economics valued at $17.5 million. Precision is eligible to receive up to $288 million in additional milestone payments based on the achievement of certain clinical, regulatory, and commercial milestones, in addition to high-single-digit to low-double-digit royalties on net sales of the licensed product.

Completed Non-Exclusive Patent License Agreement with Caribou Biosciences:

In February 2024, Precision announced that it had granted Caribou Biosciences (Nasdaq: CRBU) a non-exclusive, worldwide license, with the right to sublicense, to one of Precision’s foundational cell therapy patent families for use with CRISPR-based therapies in the field of human therapeutics. The licensed patents and applications include method and composition of matter claims that relate to Precision’s approach for targeted insertion of a sequence encoding an exogenous antigen binding receptor, such as a CAR, into the T cell receptor alpha constant (TRAC) gene locus of human T cells via a single gene edit. The licensed family, which includes more than 20 granted US and international patents, expires in October 2036.

This patent family is potentially also available for non-exclusive license to other high-quality partners in the cell therapy space.

Business Updates – $40 Million Offering:

On March 1, 2024, Precision completed a $40 million public underwritten offering consisting of 2,500,000 shares of its common stock and accompanying warrants to purchase up to 2,500,000 shares of common stock at a combined offering price of $16.00 price per share, for total gross proceeds of $40 million, before deducting underwriting discounts and commissions. The financing included participation from leading life sciences investors, including Perceptive Advisors, Janus Henderson Investors, Aquilo Capital Management, LLC and LYFE Capital.

Quarter Ended March 31, 2024 Financial Results:

Cash and Cash Equivalents: As of March 31, 2024, Precision had approximately $137.8 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. The company’s cash runway is expected to enable funding the development of our PBGENE-HBV and PBGENE-PMM *in vivo* gene editing programs through Phase 1 read out while enabling commencement of a new third wholly owned gene editing program.

Revenues: Total revenues for the quarter ended March 31, 2024, were $17.6 million, as compared to $8.8 million for the same period in 2023. The increase of $8.8 million was primarily driven by upfront investments from cell therapy transactions with TG Therapeutics and Caribou Biosciences.

Research and Development Expenses: Research and development expenses were $13.3 million for the quarter ended March 31, 2024, as compared to $11.0 million for the same period in 2023. The increase of $2.3 million was primarily due to increasing investment in our wholly owned *in vivo* gene editing programs, PBGENE-HBV and PBGENE-PMM, as the programs advance toward IND/CTA filing. The increases in program spending were partially offset by lower employee-related costs and lab supplies.

General and Administrative Expenses: General and administrative expenses were $8.4 million for the quarter ended March 31, 2024, as compared to $11.1 million for the same period in 2023. The decrease of $2.7 million was primarily related to lower employee-related costs and a reduction of external operational expenses.

Net Income/Loss from Continuing Operations: Net income from continuing operations was $8.6 million for the quarter ended March 31, 2024, inclusive of a $10.4 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 $14.0 million, for the same period in 2023. The improvement was primarily related to the revenue growth compared to the prior period as well as the non-cash gain related to the fair value adjustments of our warrant liability and Elo equity investment.

Net Loss: Net income was $8.6 million, or $1.70 per share (basic and diluted), for the quarter ended March 31, 2024, as compared to a net loss of

$25.1 million, or $(6.75) per share (basic and diluted), for the same period in 2023. The net loss in the first quarter of 2023 includes a $11.1 million loss from discontinued operations related to the decision to exit CAR T in August 2023.

Shares: Basic weighted-average common shares outstanding for the first quarter of 2024 were 5,060,978 compared to 3,709,894 for the same period in 2023. Following the common stock offering, we had 6,916,239 shares outstanding as of March 31, 2024.

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' product candidates and gene editing approaches including editing efficiency; the suitability of ARCUS nucleases for gene insertion 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 US 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 Annual Report on Form 10-K for the year ended December 31, 2023, 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 March 31,	
	2024	**2023**
Revenue	$ 17,584	$ 8,780
Operating expenses		
Research and development	13,343	11,048
General and administrative	8,428	11,086
Total operating expenses	21,771	22,134
Operating loss	(4,187)	(13,354)
Other income (expense):		
Gain (loss) from equity method investment	1,713	(1,341)
Loss on changes in fair value	(348)	(769)
Gain on change in fair value of warrant liability	10,386	—
Interest expense	(574)	(522)
Interest Income	1,663	2,043
Loss on disposal of assets	(65)	(7)
Total other income (expense)	12,775	(596)
Income (loss) from continuing operations	$ 8,588	$ (13,950)
Loss from discounted operations	—	(11,110)
Net income (loss)	$ 8,588	$ (25,060)
Net income (loss) per share		
Basic	$ 1.70	$ (6.75)
Diluted	$ 1.70	$ (6.75)
Weighted-average shares of common stock outstanding		
Basic	5,060,978	3,709,894
Diluted	5,063,406	3,709,894

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

	March 31, 2024	December 31, 2023
Cash and cash equivalents	$ 137,766	$ 116,678
Working capital	127,490	86,372
Total assets	184,741	159,781
Total liabilities	147,535	140,920
Total stockholders' equity	$ 37,206	$ 18,861
Common stock outstanding	6,916,239	4,164,038

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