Precision BioSciences Reports Second Quarter 2022 Financial Results and Provides Business Update

August 8, 2022 at 7:00 AM EDT

- Launched Exclusive In Vivo Gene Insertion Collaboration with Novartis to Develop Single ARCUS Nuclease as Potential One-time Hemoglobinopathy Treatment

- Extended Cash Runway to End of 2024

- Reported Updates Across Allogeneic CAR T Pipeline; Interim Results from Lead PBCAR0191 Study with CAR T Relapsed Subjects Who Experienced 100% Response Rate

- Published Compelling Preclinical Research in Molecular Therapy for ARCUS® In Vivo Chronic Hepatitis B Program

DURHAM, N.C.--(BUSINESS WIRE)--Aug. 8, 2022-- Precision BioSciences, Inc. (Nasdaq: DTIL), a clinical stage gene editing company developing ARCUS®-based *ex vivo* allogeneic CAR T and *in vivo* gene editing therapies, today announced financial results for the second quarter ended June 30, 2022 and provided a business update.

“This past quarter, we have executed on many important aspects of our business. From manufacturing optimization to clinical trial progress to business development and operational advancements, we are making strides towards delivering on the potential of ARCUS genome editing for drug development and adding significant cash to our balance sheet. Most exciting this past quarter, we entered into a collaboration agreement with Novartis to develop a potential one-time treatment option for hemoglobinopathies including sickle cell disease and beta thalassemia using ARCUS for gene insertion. We are very excited about expanding ARCUS into potential indications that require more complex edits, such as gene insertion,” said Michael Amoroso, Chief Executive Officer at Precision BioSciences.

“For our CAR T programs, Q2 2022 was also an exciting time for us to share encouraging new interim clinical data for our lead PBCAR0191 program. We believe the latest update validated the signal of high response rates to PBCAR0191 observed among CAR T relapsed patients we reported at the 2021 American Society for Hematology (ASH) meeting, further supporting our potential path forward in this patient population. We have recently released optimized batches of our PBCAR19B clinical trial material and look forward to commencing dosing in the next cohort of patients this quarter. In addition, we dosed PBCAR269A in combination with nirogacestat at Dose Level (DL) 3. We expect to provide additional updates across our CAR T portfolio by the end of the year,” concluded Michael Amoroso.

Recent Developments and Upcoming Milestones:

***Ex Vivo* Allogeneic CAR T Portfolio:**

PBCAR0191: PBCAR0191, azercabtagene zapreleucel (azer-cel), is Precision’s lead investigational anti-CD19 allogeneic CAR T candidate in a Phase 1/2a clinical trial of adult subjects with relapsed or refractory (R/R) non-Hodgkin lymphoma (NHL). In June 2022, Precision provided new interim data as of May 31, 2022, including high and durable clinical response rates among subjects who received a median of five prior lines of therapy. Evaluable subjects in the latest cohort of the study had the following results:

- 100% Overall Response Rate (ORR) and 73% Complete Response (CR) rate
- 50% durable response rate greater than six months
- No Grade 3 or greater cytokine release syndrome (CRS) was observed in either dosing cohort. One Grade 3 immune effector cell-associated neurotoxicity syndrome (ICANS) was recorded in each cohort that rapidly resolved to Grade 1 within 24 to 48 hours. Two Grade 5 events associated with late occurring encephalopathy suspected to be related to fludarabine-associated neurotoxicity occurred. There was no evidence of graft versus host disease.

In the second half of 2022, Precision plans to continue dosing subjects with optimized PBCAR0191 CAR T cells in this relapsed patient population while further reducing its lymphodepletion dose to standard levels.

PBCAR19B: PBCAR19B is Precision’s second generation, anti-CD19 targeting allogeneic CAR T candidate designed to evade immune rejection by host T cell and natural killer (NK) cells with a single-gene edit to knock-down beta-2 microglobulin and insert an HLA-E transgene. Manufacturing optimization for PBCAR19B was implemented in the first quarter of 2022. New clinical trial material has been released, and the company plans to commence dosing in the next cohort, DL 2 (flat dose of 540 million cells), in the third quarter of 2022.

PBCAR269A: PBCAR269A is Precision’s investigational allogeneic CAR T cell candidate targeting B-cell maturation antigen (BCMA) for R/R multiple myeloma. Precision is evaluating PBCAR269A in a Phase 1/2a study in combination with nirogacestat, a gamma secretase inhibitor (GSI) developed

by SpringWorks Therapeutics. Precision has completed DL2 (2.0×10^6 cells/kg) of PBCAR269A plus GSI and is initiating the next cohort at DL3 (flat dose of 480×10^6 cells) to further evaluate efficacy. To date, peak expansion rates observed at DL2 plus the GSI have been equivalent to DL4 (960×10^6 cells flat dose) monotherapy with no dose limiting toxicities observed.

Precision expects to provide the next update on its clinical ex vivo allogeneic CAR T programs toward year-end 2022.

***In Vivo* Gene Editing Portfolio:**

Novartis *In Vivo* Gene Editing Collaboration: In June 2022, Precision announced it had entered into an exclusive worldwide *in vivo* gene editing research and development collaboration and license agreement with Novartis. As part of the agreement, Precision will develop a single, custom ARCUS nuclease designed to insert, *in vivo*, a therapeutic transgene at a “safe harbor” location in the genome as a potential one-time transformative treatment option for diseases including certain hemoglobinopathies such as sickle cell disease and beta thalassemia. Precision will conduct *in vitro* characterization, with Novartis then assuming responsibility for all subsequent research, development, manufacturing and commercialization activities.

In addition to a $25 million equity investment from Novartis in Precision’s common stock at $2.01 per share received in June 2022, Precision received an upfront cash payment of $50 million on July 12, 2022 and is eligible to receive up to an aggregate amount of approximately $1.4 billion in additional payments for future milestones. Precision is also eligible to receive certain research funding and tiered royalties ranging from the mid-single digits to low-double digits on product sales, should Novartis successfully commercialize a therapy from the collaboration.

Lilly *In Vivo* Gene Editing Programs: Precision continues its *in vivo* gene editing collaboration with Lilly and is applying ARCUS nucleases for three initial targets, including Duchenne muscular dystrophy in muscle, a central nervous system directed target and a liver directed target.

PBGENE-PH1: Precision has initiated IND-enabling activities for its PBGENE-PH1 candidate designed to knock out the HAO1 gene as a potential one-time treatment for primary hyperoxaluria type 1 (PH1). In the first quarter of 2022, the Company initiated a non-human primate (NHP) study for PBGENE-PH1 delivered by LNP and is targeting an IND or CTA submission in 2023, subject to evaluation of data from the ongoing preclinical NHP study. Preclinical data for Precision’s PBGENE-PH1 was presented at the 2022 American Society of Gene and Cell Therapy (ASGCT) Annual Meeting and demonstrated a robust knockdown of the HAO1 protein in non-human primates following a single administration of an ARCUS nuclease via AAV.

PBGENE-HBV: Precision’s gene editing program for chronic Hepatitis B applies ARCUS to knock out persistent covalently closed circular DNA (cccDNA) and inactivate integrated hepatitis B genomes, potentially achieving durable HBV S-antigen (HBsAg) loss and reducing viral persistence. Preclinical data from this program was presented during the Gene Editing in Cancer and Complex Diseases oral session at the ASGCT Annual Meeting and within the same month published online in *Molecular Therapy* in May 2022. As reported, ARCUS efficiently targeted and degraded hepatitis B virus (HBV) cccDNA by 85% and reduced expression of HBsAg by 77% in HBV-infected primary human hepatocytes (PHH). Importantly, the optimized specificity of the ARCUS nuclease completely prevented detectable chromosomal translocations in the PHH model. PBGENE-HBV candidate is in pre-clinical development and Precision is targeting an IND/CTA submission in 2024 following completion of pre-clinical and IND enabling studies.

PBGENE-PCSK9: In 2021, Precision initiated a collaboration with iECURE, pursuant to which iECURE is expected to advance Precision’s PBGENE-PCSK9 candidate through preclinical activities as well as a Phase 1 study in familial hypercholesterolemia. As of this date, IND enabling activities for PBGENE-PCSK9 have not been completed. We are in discussions with iECURE and will provide an update on the program when more information is available.

Quarter Ended June 30, 2022 Financial Results:

Cash and Cash Equivalents: As of June 30, 2022, Precision had approximately $184.1 million in cash and cash equivalents, including receipts of $25.0 million from Novartis’ equity investment in the Company and proceeds from the June 2022 underwritten offering of the Company’s common stock, described below. The Company expects that existing cash and cash equivalents, including the cash payment of $50.0 million received from Novartis on July 12, 2022, expected operational receipts, and available credit will be sufficient to fund its operating expenses and capital expenditure requirements to the end of 2024.

In June 2022, Precision announced the closing of an underwritten offering of 35,971,224 shares of its common stock at an offering price of $1.39 per share, for total net proceeds of approximately $46.7 million, after deducting underwriting discounts and commissions and offering expenses payable by the Company.

Revenues: Total revenues for the quarter ended June 30, 2022 were $3.8 million, as compared to $68.8 million for the same period in 2021. The decrease of $65.0 million in revenue during the quarter ended June 30, 2022 was primarily the result of the absence of $62.5 million in revenue recognized under the Servier Agreement in April 2021 subsequent to full satisfaction of the performance obligation, a $1.5 million decrease in revenue recognized under the Lilly Agreement, and a $0.9 million decrease in revenue recognized from an agriculture partnering collaboration.

Research and Development Expenses: Research and development expenses were $22.9 million for the quarter ended June 30, 2022, as compared to $37.2 million for the same period in 2021. The decrease of $14.3 million was primarily due to a decrease of $11.3 million related to the Servier Program Purchase Agreement, a decrease of $2.1 million in external development costs associated with our allogeneic CAR T product candidates, a decrease of $1.3 million in employee-related costs due to reduced headcount, and a decrease of $1.7 million in clinical manufacturing organization and research costs related to our preclinical studies. These decreases were partially offset by a $1.5 million increase in sublicensing royalty payable to Duke on the Novartis upfront payment.

General and Administrative Expenses: General and administrative expenses were $10.5 million for the quarter ended June 30, 2022, as compared to $9.9 million for the same period in 2021. The increase of $0.6 million was primarily due to costs required to meet our growing infrastructure needs, including consulting fees and employee-related costs associated with increased share-based compensation expense.

Net Loss: Net loss was $31.0 million, or $(0.46) per share (basic and diluted), for the quarter ended June 30, 2022, as compared to net income of $21.7 million, or $0.38 per share (basic) and $0.36 per share (diluted), for the same period in 2021.

Corporate:

Executive Leadership: In May 2022, Juli Blanche was appointed Chief People Officer and a member of the senior leadership team. Ms. Blanche joined Precision from Bristol-Myers Squibb where she was Senior Vice President and HR Business Partner, Global Commercialization. She brings over 25 years of strategic business partnership and enterprise leadership experience in the pharmaceutical and financial services industries.

Board of Directors: In May 2022, Melinda Brown was appointed as a Director on Precision's Board of Directors and Chair of the Board's Audit Committee. Ms. Brown is a financial expert with proven experience leading accounting, finance and enterprise risk management teams in large, public companies, including Tapestry, Inc. and PepsiCo, Inc.

Together, Ms. Blanche and Ms. Brown provide substantial executive leadership that will be paramount for Precision as the company embarks upon the next phase of its life cycle.

About Precision BioSciences, Inc.

Precision BioSciences, Inc. is a clinical stage biotechnology company dedicated to improving life (DTIL) with its novel and proprietary ARCUS® genome editing platform. ARCUS is a highly precise and versatile genome editing platform that was designed with therapeutic safety, delivery, and control in mind. Using ARCUS, the company's pipeline consists of multiple *ex vivo* "off-the-shelf" CAR T immunotherapy clinical candidates and several *in vivo* gene editing candidates designed to cure 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 efficacy and benefit of our product candidates, benefits of ARCUS and potential expansion and development using ARCUS, the expected timing of updates regarding our allogeneic CAR T and *in vivo* programs, the expected timing of communications with regulators, the expected advancement toward and timing of IND and CTA filings, the ability of our product candidates, if approved, to become best-in-class or first-in-class, the planned development activities with our collaboration partners, expectations about our operational initiatives and business strategy, achieving key milestones and additional collaborations, and expectations regarding our liquidity and ability to fund operating expenses and capital expenditures requirements. In some cases, you can identify forward-looking statements by terms such as "aim," "anticipate," "approach," "believe," "contemplate," "could," "estimate," "expect," "goal," "intend," "look," "may," "mission," "plan," "possible," "potential," "predict," "project," "should," "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. Such statements are subject to 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 and requirements under our current debt instruments and effects of restrictions thereunder; risks associated with raising additional capital; our operating expenses and our ability to predict what those expenses will be; our limited operating history; the 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; our dependence on our ARCUS technology; the initiation, cost, timing, progress, achievement of milestones and results of research and development activities, preclinical studies and clinical trials; 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 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 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; our or our collaborators' ability to advance product candidates into, and successfully design, implement and complete, clinical or field trials; potential manufacturing problems associated with the development or commercialization of any of our product candidates; our ability to obtain an adequate supply of T cells from qualified donors; our ability to achieve our anticipated operating efficiencies at our manufacturing facility; 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; the rate and degree of market acceptance of any of our product candidates; 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; our ability to effectively manage the growth of our operations; our ability to attract, retain, and motivate key executives and personnel; market and economic conditions; effects of system failures and security breaches; effects of natural and manmade disasters, public health emergencies and other natural catastrophic events; effects of COVID-19 pandemic and variants thereof, or any pandemic, epidemic or outbreak of an infectious disease; insurance expenses and exposure to uninsured liabilities; effects of tax rules; risks related to ownership of our common stock and other important factors discussed under the caption "Risk Factors" in our Annual Report on Form 10-K for the fiscal year ended December 31 2021, as any such factors may be updated from time to time in our other filings with the SEC, including, but not limited to, our Quarterly Report on Form 10-Q for the quarterly period ended June 30, 2022, to be filed 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 Consolidated Statements of Operations

(In thousands, except share and per share amounts)

(unaudited)

	For the Three Months Ended June 30,	
	2022	**2021**
Revenue	$ 3,820	$ 68,805
Operating expenses		
Research and development	22,936	37,235
General and administrative	10,485	9,938
Total operating expenses	33,421	47,173
Operating (loss) income	(29,601)	21,632
Other (expense) income:		
Loss from equity method investment	(1,448)	—
Interest expense	(178)	(24)
Interest income	192	48
Total other (expense) income, net	(1,434)	24
Net (loss) income	$ (31,035)	$ 21,656
Net (loss) income per share		
Basic	$ (0.46)	$ 0.38
Diluted	$ (0.46)	$ 0.36
Weighted average shares of common stock outstanding		
Basic	67,954,688	57,739,622
Diluted	67,954,688	59,841,638

	For the Six Months Ended June 30,	
	2022	**2021**

Revenue	$ 7,137	$ 85,154
Operating expenses		
Research and development	42,908	62,828
General and administrative	21,176	19,436
Total operating expenses	64,084	82,264
Operating (loss) income	(56,947)	2,890
Other (expense) income:		
Loss from equity method investment	(2,400)	—
Interest expense	(220)	(24)
Interest income	364	101
Total other (expense) income, net	(2,256)	77
Net (loss) income	$ (59,203)	$ 2,967
Net (loss) income per share		
Basic	$ (0.92)	$ 0.05
Diluted	$ (0.92)	$ 0.05
Weighted average shares of common stock outstanding		
Basic	64,512,356	57,185,402
Diluted	64,512,356	59,647,367

Precision Biosciences, Inc.

Condensed Consolidated Balance Sheets Data

(In thousands)

(Unaudited)

	June 30, 2022	**December 31, 2021**
Cash and cash equivalents	$ 184,135	$ 143,663

Working capital	193,769	125,774
Total assets	297,163	211,498
Total stockholders' equity	$ 103,023	$ 91,168

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