# Precision BioSciences Reports Third Quarter 2021 Financial Results and Provides Business Update

November 10, 2021

*- Appointed Michael Amoroso as President and Chief Executive Officer and to Board of Directors; Sam Wadsworth, Ph.D., and Shari Lisa Piré, J.D., Appointed to Board of Directors*

*- Hosted In Vivo Gene Editing R&D Event and Outlined Plans for Accelerated Path to Clinic for Three Investigational New Drug/Clinical Trial Applications Within Next Three Years*

*- Announced In Vivo Gene Editing License and Collaboration Agreement with iECURE to Advance Precision BioSciences' Wholly-Owned PCSK9 Program for Familial Hypercholesterolemia Through Phase 1 and Develop Four ARCUS-Based Gene Insertion Programs*

*- Company to Present Interim Data from PBCAR0191 Allogeneic CAR T Program During Two Oral Presentations at Upcoming American Society of Hematology Annual Meeting; Company to Host Webcast on Saturday, December 11, 2021 to Provide a CAR T Portfolio Update*

DURHAM, N.C.--(BUSINESS WIRE)--Nov. 10, 2021-- Precision BioSciences, Inc. (Nasdaq: DTIL), a clinical stage biotechnology company using its ARCUS® genome editing platform to develop allogeneic CAR T and *in vivo* gene editing therapies, today announced financial results for the third quarter ended September 30, 2021 and provided a business update.

"Precision is dedicated to delivering on the vast potential of ARCUS gene editing for best-in-class cell and gene therapeutics. In addition to significant research and development progress this past quarter, we signed strategic license and development agreements that we believe are accelerating certain *in vivo* program timelines to the clinic and providing us with important optionality for future product development efforts," said Michael Amoroso, Chief Executive Officer at Precision BioSciences.

"During our *in vivo* gene editing event in September, we showcased data supporting ARCUS' differentiating capabilities for gene knockout, gene insertion and gene repair, and mitochondrial DNA gene editing. We also outlined an *in vivo* gene editing clinical development strategy designed to advance three investigational new drug (IND) or clinical trial applications (CTA) over the next three years. In addition, we continue to actively recruit patients for our ongoing clinical studies with PBCAR0191, PBCAR19B, and PBCAR269A while monitoring for safety, overall response rate, and durability of response. We look forward to sharing updated data on the PBCAR0191 program during two oral presentations at the upcoming American Society of Hematology conference," Mr. Amoroso continued.

**Recent Developments and Upcoming Milestones:**

**Allogeneic CAR T Portfolio:** Precision BioSciences has treated more than 100 patients with PBCAR T cells across its clinical CAR T programs. Precision's CAR T cells are the only allogeneic CAR T cells in human clinical trials made with a single gene editing step designed to specifically avoid potentially deleterious off target editing effects and preserve cell health and viability.

**PBCAR0191:** Precision continues to enroll patients in its Phase 1/2a study of PBCAR0191, targeting CD19 for the treatment of Relapsed/Refractory (R/R) non-Hodgkin lymphoma (NHL) or R/R B-cell precursor acute lymphoblastic leukemia (B-ALL). New interim data for PBCAR0191 will be presented at the upcoming 63$^{rd}$ American Society of Hematology Annual Meeting. Oral presentations will be delivered by Bijal Shah, M.D., from Moffitt Cancer Center and Nitin Jain, M.D., from The University of Texas MD Anderson Cancer Center on Saturday, December 11, 2021 and Monday, December 13, 2021, respectively. Precision will host a webcast on Saturday, December 11, 2021 to provide a CAR T portfolio update.

**PBCAR19B:** In July 2021, Precision announced that the first patient was dosed in its Phase 1 clinical study of PBCAR19B, the Company's anti-CD19 immune-evading stealth cell candidate for the treatment of R/R NHL. PBCAR19B is being administered at flat dose levels starting at $2.7 \times 10^8$ CAR T cells per patient, the same as Dose Level 3 for PBCAR0191. Precision expects to provide an initial study update in 2022.

**PBCAR269A:** PBCAR269A is an investigational allogenic CAR T immunotherapy targeting B-cell maturation antigen (BCMA) for the treatment of R/R multiple myeloma. Precision continues to enroll patients in the combination arm of its Phase 1/2a study of evaluating PBCAR269A with nirogacestat, a gamma secretase inhibitor developed by SpringWorks Therapeutics. Precision expects to provide an interim update on the monotherapy arm of the study during its conference webcast on December 11, 2021.

**PBCAR20A:** PBCAR20A is an investigational allogeneic CAR T cell immunotherapy targeting CD20 for the treatment of R/R NHL in a heterogeneous population who were all previously treated with anti-CD20 monoclonal antibodies. Treatment with PBCAR20A did not result in compelling response rates in a Phase 1/2a clinical study. While this study provided important information regarding allogeneic CAR T dosing and lymphodepletion regimens, Precision has decided to focus its clinical efforts in R/R lymphoma on CD19 targeting programs, as CD19 is a more robust antigenic target in R/R heterogeneous NHL populations. Precision will not continue development of PBCAR20A. All subjects enrolled in the study and evaluated for treatment with PBCAR20A had acceptable tolerability with no graft versus host disease, no Grade ≥ 3 cytokine release syndrome, and no Grade ≥ 3 neurotoxicity (ICANS).

**Tiziana:** In September 2021, Precision signed an exclusive license agreement to evaluate Tiziana's foralumab, a fully human anti-CD3 monoclonal antibody, as a lymphodepleting agent in conjunction with Precision's allogeneic CAR T cells for the potential treatment of cancers. This agreement reflects Precision's ongoing pursuit of a best-in-class allogeneic CAR T cell therapy.

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

**Accelerated Research & Development Strategy:** At the Company's *in vivo* gene editing R&D event on September 9, 2021, Precision outlined its clinical development strategy and plans to accelerate three programs to human clinical trials: familial hypercholesterolemia (FH) as early as 2022, primary hyperoxaluria type 1 (PH1) in 2023, and chronic hepatitis B virus (HBV) in 2024. The event featured preclinical data differentiating the precision, safety and versatility of ARCUS, including the first presentation of ARCUS-mediated gene insertion in non-human primates (NHPs) and mitochondrial DNA gene editing. Specific program updates featured the following:

- **PBGENE-PCSK9 & iECURE Collaboration:** In August 2021, Precision signed a license and collaboration agreement with iECURE, a mutation-agnostic *in vivo* gene editing company co-founded by James M. Wilson, M.D., Ph.D. Under the agreement, iECURE will advance Precision's wholly-owned PBGENE-PCSK9 candidate into a Phase 1 study in FH, with a CTA filing expected as early as 2022. iECURE will also use Precision's PCSK9-directed ARCUS nuclease to develop four other pre-specified gene insertion therapies for rare genetic diseases, including ornithine transcarbamylase (OTC) deficiency (iECURE-OTC), phenylketonuria (iECURE-PKU) and two other programs focused on liver diseases. Precision received an equity stake in iECURE and is eligible to receive milestone and mid-single digit to low double digit royalty payments on sales of iECURE products developed with ARCUS.

- **PBGENE-PH1:** Preclinical research continues to progress for Precision's wholly-owned *in vivo* gene editing program applying ARCUS to knock out the HAO1 gene as a potential one-time treatment for PH1. In September 2021, Precision presented NHP data showing on average, a 98.0% reduction in HAO1 mRNA and a 97.9% reduction in the encoded protein after a single administration of an AAV vector encoding ARCUS. Precision has initiated IND-enabling activities and expects to submit an IND/CTA in 2023 for PBGENE-PH1 delivered by lipid nanoparticle.

- **PBGENE-HBV:** Precision's gene editing program for chronic HBV applies ARCUS to knock out persistent closed circular DNA (cccDNA) and potentially reduce viral persistence. Previously reported preclinical data has shown that ARCUS efficiently targeted and degraded HBV cccDNA in HBV-infected primary human hepatocytes and reduced expression of HBV S-antigen (HBsAg) by as much as 95%. Similar levels of HBsAg reduction were observed in a newly developed mouse model of HBV infection following administration of ARCUS mRNA using lipid nanoparticle delivery. Precision expects to submit an IND/CTA in 2024.

- **Lilly *In Vivo* Gene Editing Programs:** Programs announced as part of the research and development agreement with Lilly, including Duchenne muscular dystrophy (PBGENE-DMD), a liver-directed target (PBGENE-LLY2) and a CNS-directed target (PBGENE-LLY3) continue to progress.

**Corporate:**

**Executive Leadership:** In September 2021, Michael Amoroso was appointed President and Chief Executive Officer and a member of Precision's Board of Directors, both effective October 15, 2021. Mr. Amoroso joined Precision from Abeona Therapeutics, where he served most recently as Chief Executive Officer. He succeeded Matt Kane, Co-Founder, President, and Chief Executive Officer who is serving as an advisor to the Company.

**Board Appointments:** On November 8, 2021, Sam Wadsworth, Ph.D., Chief Scientific Officer at Ultragenyx Gene Therapy, an operating unit of Ultragenyx Pharmaceutical Inc., and Shari Lisa Piré, J.D., Chief Legal & Sustainability Officer at Plume Design were appointed to the Company's Board of Directors. Together Dr. Wadsworth and Ms. Piré add significant cell and gene therapy drug development and strategic business growth experience.

**Elo Life Systems:**

**Corporate Structure:** In January 2021, Precision BioSciences disclosed its intention to spinout Elo Life Systems, a wholly-owned subsidiary of Precision. Precision continues to explore strategic options and expects to complete any such spinout, sale or other treatment of Elo in 2021.

**Quarter Ended September 30, 2021 Financial Results**

**Cash and Cash Equivalents:** As of September 30, 2021, Precision had approximately $160.5 million in cash and cash equivalents. The Company expects that existing cash and cash equivalents, expected operational receipts, and available credit will be sufficient to fund its operating expenses and capital expenditure requirements into 2023.

**Revenues:** Total revenues for the third quarter ended September 30, 2021 were $24.0 million, as compared to $7.4 million for the same period in 2020. The increase of $16.6 million in revenue during the three months ended September 30, 2021 was primarily the result of an $17.9 million in revenue recognized under the iECURE agreement as the agreement was executed in August 2021. In the third quarter of 2021, the Company also recognized $4.9 million in revenue under the Lilly Agreement, as work commenced in 2021. These increases in revenue were partially offset by a $7.0 million decrease in revenue recognized under the Servier Agreement compared to the third quarter of 2020, as the performance obligation was deemed fully satisfied upon the execution of the Program Purchase Agreement in April 2021.

**Research and Development Expenses:** Research and development expenses were $25.9 million for the quarter ended September 30, 2021, as compared to $24.9 million for the same period in 2020.

**General and Administrative Expenses:** General and administrative expenses were $9.6 million for the quarter ended September 30, 2021, as compared to $8.5 million for the same period in 2020. The increase was primarily due to costs required to meet our growing infrastructure needs, including an increase of $1.2 million in general and administrative employee-related costs.

**Net Loss:** Net loss was $11.3 million, or $(0.19) per share (basic and diluted), for the quarter ended September 30, 2021, as compared to a net loss of $26.0 million, or $(0.50) per share (basic and diluted), for the same period in 2020.

**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 specific 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 "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 of our product candidates, including the expected timing of further interim updates regarding PBCAR0191, PBCAR19B, and PBCAR269A, the expected timing of future IND and CTA filings, the potential clinical benefit of our allogeneic CAR T product candidates, the planned development activities pursuant to our agreement with Tiziana, the planned development activities pursuant to our agreement with iECURE, the potential value of iECURE equity, any future milestones or royalty payments under our development and collaboration agreements, the development and commercial potential of ARCUS-mediated gene addition and developments related to our expected spinout or other treatment of Elo Life Systems, our expected participation in future industry events and conferences and expectations about our operational initiatives and business strategy. In some cases, you can identify forward-looking statements by terms such as "aim," "anticipate," "believe," "could," "expect," "should," "plan," "intend," "estimate," "target," "mission," "goal," "may," "will," "would," "should," "could," "target," "potential," "project," "predict," "contemplate," "potential," 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 or greenhouse studies and clinical or field trials; public perception about genome editing technology and its applications; competition in the genome editing, biopharmaceutical, biotechnology and agricultural 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 the outbreak of COVID-19, 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 Quarterly Report on Form 10-Q for the quarterly period ended June 30, 2021, as any such factors may be updated from time to time in our other filings with the SEC, including our Quarterly Report on Form 10-Q for the quarterly period ended September 30, 2021, which are accessible on the SEC's website at www.sec.gov and the Investors page of our website under Events & Presentations 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 September 30, | |
|---|---|---|
| | **2021** | **2020** |
| Revenue | $ 24,036 | $ 7,363 |
| Operating expenses | | |

| | | |
|---|---|---|
| Research and development | 25,940 | 24,873 |
| General and administrative | 9,638 | 8,534 |
| Total operating expenses | 35,578 | 33,407 |
| Operating loss | (11,542) | (26,044) |
| Other income (expense): | | |
| Change in fair value of investments | 274 | - |
| Interest expense | (55) | - |
| Interest income | 44 | 28 |
| Total other income, net | 263 | 28 |
| Net loss and net loss attributable to common stockholders | $ (11,279) | $ (26,016) |
| Net loss per share attributable to common stockholders - basic and diluted | $ (0.19) | $ (0.50) |
| Weighted average shares of common stock outstanding - basic and diluted | 59,657,677 | 52,346,715 |

| | **For the Nine Months Ended September 30,** | |
|---|---|---|
| | **2021** | **2020** |
| Revenue | $ 109,190 | $ 15,439 |
| Operating expenses | | |
| Research and development | 88,768 | 74,935 |
| General and administrative | 29,074 | 26,852 |
| Total operating expenses | 117,842 | 101,787 |
| Operating loss | (8,652) | (86,348) |
| Other income (expense): | | |
| Change in fair value of investments | 274 | - |
| Interest expense | (79) | - |
| Interest income | 145 | 795 |
| Total other income, net | 340 | 795 |
| Net loss and net loss attributable to common stockholders | $ (8,312) | $ (85,553) |
| Net loss per share attributable to common stockholders - basic and diluted | $ (0.14) | $ (1.65) |
| Weighted average shares of common stock outstanding - basic and diluted | 58,018,550 | 51,858,032 |

**Precision Biosciences, Inc.**
**Condensed Consolidated Balance Sheets Data**
(In thousands)
(Unaudited)

| | September 30, 2021 | December 31, 2020 |
|---|---|---|
| Cash and cash equivalents | $ 160,471 | $ 89,798 |
| Working capital | 146,573 | 62,735 |
| Total assets | 227,199 | 150,158 |
| Total stockholders' equity | $ 101,443 | $ 44,425 |

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