# Precision BioSciences Reports Fourth Quarter and Fiscal Year 2021 Financial Results and Provides Business Update

March 15, 2022

*- Advancing ARCUS® Platform by Developing In Vivo Gene Editing Programs for Genetic Diseases and Ex Vivo CAR T Therapies for Hematologic Malignancies*

*- Presented Allogeneic CAR T Clinical Data at the American Society of Hematology Meeting Showing High Response Rates to PBCAR0191 in Heavily Treated NHL and B-ALL Patients Who Relapsed Following Prior Autologous CAR T Therapy*

*- Progressing Ex Vivo Allogeneic CAR T Programs with Updates Expected in Mid-2022*

*- Advancing Three In Vivo Gene Editing Programs to IND or CTA in the Next Three Years*

*- Focusing Investment on Human Therapeutics Following Spin-Out of Elo Life Systems*

DURHAM, N.C.--(BUSINESS WIRE)--Mar. 15, 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 fourth quarter and fiscal year ended December 31, 2021.

“Precision BioSciences made significant progress in 2021 toward validating our differentiated ARCUS gene editing platform to improve human health. Our team advanced two *ex vivo* CAR T product candidates targeting CD19 with the goal of developing a potential first-in-class and a potential best-in-class allogeneic CAR T treatment, if approved. We began operationalizing two key strategic partnerships, including our transformational *in vivo* gene editing alliance with Lilly covering up to six gene targets, and a partnership with iECURE targeting expedited clinical development of our wholly owned PBGENE-PCSK9 program. Under the iECURE agreement we expect ARCUS and its unique properties to be leveraged in four gene insertion programs for rare diseases,” said Michael Amoroso, Chief Executive Officer at Precision BioSciences. “Finally, in late 2021, we completed the spin-out of our food and agriculture business to allow our team to focus solely on human therapeutics.”

“Looking ahead to the remainder of 2022 and beyond, we intend to continue building on our operational effectiveness and take the next steps to further validate ARCUS clinically. In addition to planned updates for our ongoing allogeneic CAR T clinical studies with PBCAR0191, PBCAR19B, and PBCAR269A in 2022, we also plan to advance three wholly owned *in vivo* gene editing programs to investigational new drug applications (IND) or clinical trial applications (CTA) over the next three years,” Mr. Amoroso continued.

**Recent Developments and Upcoming Milestones:**

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

Precision’s CAR T cells are the only allogeneic CAR T cells in human clinical trials made with a single-gene editing step, enabled by ARCUS, which is specifically designed to avoid potentially deleterious off target editing effects and preserve cell health and viability.

**PBCAR0191:** Precision presented updated interim data on its Phase 1/2a study of PBCAR0191 with enhanced lymphodepletion[1] (eLD) at the 63rd American Society of Hematology Annual Meeting, which included 22 heavily pre-treated relapsed/refractory subjects with predominantly advanced or aggressive B-cell malignancies who received a median five lines of prior treatment, including 27% (6/22) who previously received a CD19-directed autologous CAR T product and progressed.

For patients that received treatment of PBCAR0191 following eLD as of November 16, 2021:

- PBCAR0191 showed no ≥ Grade 3 cytokine release syndrome (CRS), one Grade 3 immune effector cell-associated neurotoxicity syndrome (ICANS) with resolution to ≤ Grade 2 in 72 hours, no evidence of graft-versus-host disease, and one infectious death at Day 54 deemed possibly related to treatment.
- PBCAR0191 yielded an overall response rate (ORR) of 73% and a complete response rate (CR) of 59% using a single dose of $3 \times 10^6$ cells/kg at ≥Day 28.
- Four responders among the 17 evaluable non-Hodgkin’s lymphoma (NHL) subjects reached Day 180 durability assessment with a single dose.

Most notably, a potential signal for PBCAR0191 was observed among six subjects that previously received an autologous CAR T therapy and progressed:

- These subjects experienced an ORR of 100% and a CR of 66% at ≥Day 28.
- More than half of these subjects had a longer duration of response on PBCAR0191 than with the prior autologous CAR T

treatment.

Precision BioSciences is prioritizing enrollment of these high unmet need NHL patients who have relapsed after receiving an autologous CAR T therapy as a potential path for PBCAR0191 to be a potential first-in-class allogeneic CAR T therapy.

**PBCAR19B:** PBCAR19B is a novel, immune-evading stealth cell candidate employing a single-gene edit to knock-down beta-2 microglobulin and express an HLA-E transgene. PBCAR19B is the first CAR T cell candidate in the clinic designed to evade rejection by host T cells and natural killer (NK) cells. Precision initiated a clinical trial of PBCAR19B in patients with NHL in mid-2021 and completed dosing at Dose Level 1. Precision plans to commence dosing at the next dose level with clinical trial material from an optimized manufacturing process once released and expects to provide a program update in mid-2022.

**CD19 Combination with Foralumab:** Precision also plans to evaluate one of its anti-CD19 CAR T cell candidates in combination with foralumab, a fully humanized anti-CD3 antibody from Tiziana Life Sciences, and expects to update its IND in 2022 to enable combination use. Including an anti-CD3 antibody as part of the lymphodepletion regimen may further reduce CAR T cell rejection by targeting CD3+ host T cells and suppressing their anti-CAR T response, enabling the CAR T cells to expand, proliferate, and persist to maximize long term clinical benefits.

**PBCAR269A:** PBCAR269A is an investigational allogenic 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 developed by SpringWorks Therapeutics and expects to provide an update in mid-2022.

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

With respect to its own wholly owned organic pipeline, Precision expects that three of its preclinical *in vivo* programs will advance to IND or CTA in the next three years:

- **PBGENE-PCSK9**: In 2021, Precision initiated a collaboration with iECURE, a mutation-agnostic *in vivo* gene editing company co-founded by James M. Wilson, M.D., Ph.D., pursuant to which Precision's wholly owned PBGENE-PCSK9 candidate is expected to be advanced through preclinical activities as well as a Phase 1 study in familial hypercholesterolemia. Long-term durability and safety of ARCUS *in vivo* gene editing to cut LDL cholesterol levels in nonhuman primates (NHPs) has been published in *Nature Biotechnology* (July 2018) and *Molecular Therapy* (June 2021). Nearly five years later, NHPs in this 2017 study continue to be monitored for ongoing, sustained reduction of LDL cholesterol levels while maintaining stable gene editing and data from these trials has not shown any obvious adverse events to date. A CTA filing is expected as early as the end of 2022. iECURE also expects to use an ARCUS nuclease to develop gene insertion programs in four rare genetic diseases.
- **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 primary hyperoxaluria type 1 (PH1). Precision has initiated IND-enabling activities and expects to submit an IND or CTA in 2023 for PBGENE-PH1 delivered by lipid nanoparticle (LNP).
- **PBGENE-HBV:** Precision's gene editing program for chronic HBV applies ARCUS to knock out persistent closed circular DNA (cccDNA) and inactivate integrated hepatitis B genomes, potentially achieving durable HBV S-antigen (HBsAg) loss and viral clearance. Previously reported preclinical data has shown that ARCUS efficiently targeted and degraded HBV cccDNA in HBV-infected primary human hepatocytes and reduced expression of HBsAg by as much as 95%. Utilizing newly developed models of HBV infection, high levels of ARCUS gene editing were demonstrated in both mice and non-human primates after LNP administration of ARCUS mRNA. In the HBV mouse model, which sustains HBsAg expression, ARCUS gene editing resulted in >95% HBsAg reduction. Precision will pursue clinical development of its PBGENE-HBV candidate using LNP delivery and expects to submit an IND/CTA in 2024.

In 2021, Precision began an *in vivo* gene editing collaboration with Lilly and made progress applying ARCUS nucleases for three initial targets, including Duchenne muscular dystrophy in muscle, a central nervous system directed target, and a liver directed target.

**Corporate:**

In December 2021, Precision announced its entry into an agreement with a syndicate of investors led by ACCELR8 to separate its wholly owned Elo Life Systems subsidiary and create an independent food and agriculture business, which is intended to enable us to focus exclusively on human therapeutics.

**Fiscal Year 2021 Financial Results:**

**Cash and Cash Equivalents:** As of December 31, 2021, Precision had approximately $143.7 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 mid-2023.

**Revenues:** Total revenues for the year ended December 31, 2021 were $115.5 million, as compared to $24.3 million for the same period in 2020. The increase of $91.2 million in revenue during the year ended December 31, 2021 was primarily the result of a $54.8 million increase in revenue recognized under the Servier Agreement as the performance obligation was deemed fully satisfied upon the execution of the Program Purchase Agreement with Servier, a $21.0 million increase in revenue recognized under the Lilly Agreement as work began in 2021, a $17.9 million increase in revenue recognized under the iECURE Agreement which was executed in 2021, and a $2.9 million increase in revenue recognized from an agricultural partnering collaboration.

**Research and Development Expenses:** Research and development expenses were $115.2 million for the year ended December 31, 2021, as

compared to $98.1 million for the same period in 2020. The increase of $17.1 million in research and development expenses was primarily due to a $11.3 million increase in expenses related to the Servier Program Purchase Agreement, pursuant to which the Company reacquired all of its global development and commercialization rights related to *ex vivo* allogeneic CAR T targets previously named by Servier pursuant to the Servier Development and Commercial License Agreement.

**General and Administrative Expenses:** General and administrative expenses were $39.7 million for the year ended December 31, 2021, as compared to $36.1 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 $3.9 million in general and administrative employee-related costs associated with increased wages, share-based compensation, and recruiting costs for key management personnel.

**Net Loss:** Net loss was $30.6 million, or $(0.52) per share (basic and diluted), for the year ended December 31, 2021, as compared to a net loss of $109.0 million, or $(2.09) 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 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 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 status and potential clinical benefit of our allogeneic CAR T product candidates, including the potential of our product candidates, if approved, to become best-in-class or first-in-class, expected IND updates and advancement of preclinical programs to IND or CTA, the planned development activities pursuant to our agreement with Tiziana, the planned development activities pursuant to our agreement with iECURE, our expected participation in future industry events and conferences expectations about our operational initiatives and business strategy, 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," "believe," "best-in-class", "contemplate," "could," "estimate," "expect," "first-in-class," "goal," "intend," "may," "mission," "plan," "predict," "potential," "look", "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 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 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 Quarterly Report on Form 10-Q for the quarterly period ended September 30, 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 Annual Report on Form 10-K for the fiscal year ended December 31, 2021, 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.**

**Consolidated Statements of Operations**

(In thousands, except share and per share amounts)

| | For the Years Ended December 31, | |
|---|---|---|
| | **2021** | **2020** |
| Revenue | $ 115,529 | $ 24,285 |
| Operating expenses | | |
| Research and development | 115,238 | 98,061 |
| General and administrative | 39,693 | 36,052 |
| Total operating expenses | 154,931 | 134,113 |
| Operating loss | (39,402) | (109,828) |
| Other income (expense): | | |
| Gain on changes in fair value | 2,555 | — |
| Gain on deconsolidation of subsidiary | 5,985 | — |
| Income from equity method investments | 184 | — |
| Interest expense | (132) | — |
| Interest income | 208 | 822 |
| Total other income, net | 8,800 | 822 |
| Net loss and net loss attributable to common stockholders | $ (30,602) | $ (109,006) |
| Net loss per share attributable to common stockholders - basic and diluted | $ (0.52) | $ (2.09) |
| Weighted average shares of common stock outstanding - basic and diluted | 58,688,102 | 52,031,740 |

**Precision Biosciences, Inc.**

**Consolidated Balance Sheets Data**

(In thousands)

| | December 31, 2021 | December 31, 2020 |
|---|---|---|
| Cash and cash equivalents | $ 143,663 | $ 89,798 |
| Working capital | 125,774 | 62,735 |
| Total assets | 211,498 | 150,158 |
| Total stockholders' equity | $ 91,168 | $ 44,425 |

[1]Enhanced lymphodepletion = Fludarabine (30 mg/m $^2$/day × 4 days) and cyclophosphamide (1000 mg/m $^2$/day × 3 days)

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**Investor Contact:**
Alex Kelly
Chief Financial Officer
Alex.Kelly@precisionbiosciences.com

**Media Contact:**
Maurissa Messier
Senior Director, Corporate Communications
Maurissa.Messier@precisionbiosciences.com

Source: Precision BioSciences, Inc.