# Precision BioSciences Reports Fourth Quarter and Fiscal Year 2020 Financial Results and Provides Business Update

March 18, 2021

*Established in vivo genome editing research collaboration and exclusive license agreement with Eli Lilly*

*Reported interim data from Phase 1/2a study of PBCAR0191; updated interim data expected by mid-year 2021*

*Interim data releases expected from PBCAR269A and PBCAR20A in 2021*

*Received FDA acceptance of IND application for PBCAR19B, a next-generation, stealth cell, anti-CD19 allogeneic CAR T candidate for non-Hodgkin lymphoma; Phase 1 study expected to be initiated by mid-year 2021*

DURHAM, N.C., March 18, 2021 (GLOBE NEWSWIRE) -- Precision BioSciences, Inc. (Nasdaq: DTIL) a clinical stage biotechnology company dedicated to improving life with its proprietary ARCUS® genome editing platform, today announced financial results for the fourth quarter and fiscal year ended December 31, 2020 and provided a business update.

"2020 was a pivotal year for Precision with significant advances across our ARCUS-based *in vivo* gene editing and allogeneic CAR T pipelines. We entered into a research collaboration and exclusive license agreement with Eli Lilly for the research and development of multiple *in vivo* gene correction therapies aimed at potentially curing genetic disorders, including Duchenne muscular dystrophy. In addition to advancing two new CAR T programs into the clinic, we reported interim study results for our lead CD19 program, which showed continued acceptable safety alongside high objective response rates," commented Matt Kane, CEO and co-founder of Precision BioSciences. "In 2021, we look forward to starting our Phase 1/2a study of PBCAR19B to assess whether our next-generation, stealth, donor CAR T cells can persist longer in the body, a key goal that we believe could lead to deep and durable responses with off-the-shelf CAR T products. We also expect to report in 2021 interim data updates for our three clinical CAR T programs, PBCAR0191, PBCAR20A, and PBCAR269A, and provide an update on our PH1 *in vivo* gene editing program."

**Recent Developments and Upcoming Milestones:**

**Allogeneic CAR T Portfolio:**

**PBCAR0191:** In December 2020, Precision reported encouraging interim clinical results for its Phase 1/2a study of patients with relapsed/refractory (R/R) non-Hodgkin lymphoma (NHL) and R/R B-cell Acute Lymphoblastic leukemia (B-ALL). For this study, 27 patients across multiple dose levels received PBCAR0191 with either standard lymphodepletion (sLD) (fludarabine 30 mg/m$^2$/day x 3 days + cyclophosphamide 500 mg/m$^2$/day x 3 days) or enhanced lymphodepletion (eLD) (fludarabine 30 mg/m$^2$/day x 4 days + cyclophosphamide 1000 mg/m$^2$/day x 3 days). Response rates were as follows:

- 83% objective response rate at day 28 or later for patients across NHL (n=4) and B-ALL (n=2) who received PBCAR0191 when coupled with eLD.
- At day 28 or later, 75% (3/4) of NHL patients who received PBCAR0191 with eLD achieved a complete response compared to 33% of NHL patients (n=9) using sLD.
- The longest demonstrated response was > 11 months in a B-ALL patient.

PBCAR0191 demonstrated a clear dose-dependent increase in peak cell expansion. Compared to sLD, eLD with PBCAR0191 resulted in approximately 95-fold increase in peak cell expansion, and approximately 45-fold increase in area under the curve. PBCAR0191 demonstrated an acceptable safety profile, with no graft versus host disease (GvHD), no grade ≥ 3 cytokine release syndrome, and no grade ≥ 3 neurotoxicity (ICANS).

Precision expects to provide updated interim data for PBCAR0191 by mid-2021.

**PBCAR19B:** In January 2021, Precision announced that the U.S. Food and Drug Administration (FDA) accepted its investigational new drug application for PBCAR19B, the Company's next generation, stealth cell, allogeneic CAR T candidate for patients with CD19-positive malignancies such as those with R/R NHL. PBCAR19B is designed to improve the persistence of allogeneic CAR T cells following infusion by reducing rejection by T cells and natural killer (NK) cells. In addition to the CAR gene, the PBCAR19B vector includes a short hairpin RNA that suppresses expression of beta-2 microglobulin (B2M), a component of Class 1 major histocompatibility complex (MHC) molecules found on the cell surface. Reducing or knocking down Class 1 MHC expression on allogeneic CAR T cells has been shown to reduce CAR T cell killing by cytotoxic T cells. The PBCAR19B vector also carries an HLA-E gene intended to reduce rejection of CAR T cells by NK cells that can be stimulated as a result of reduced MHC molecule expression on the cell surface.

The Phase 1 study is expected to begin by mid-year 2021 and will be a non-randomized, open-label, single-dose, dose-escalation and dose-expansion study to evaluate the safety and clinical activity of PBCAR19B in patients with R/R NHL. The primary objective of the study is to identify the maximum tolerated dose and any dose-limiting toxicities.

**PBCAR20A:** Precision also continues to enroll patients in its Phase 1/2a clinical trial of PBCAR20A, a wholly-owned investigational allogeneic anti-CD20 CAR T therapy for patients with R/R NHL including patients with R/R chronic lymphocytic leukemia or R/R small lymphocytic lymphoma. In February 2021, the study began enrolling patients into dose level 3, a fixed dose of 480 x $10^6$ cells with a max dose of 6.0 × $10^6$ cells/kg. The Company expects to report interim data for the PBCAR20A study in 2021.

**PBCAR269A:** Precision continues to enroll patients in its Phase 1/2a study of PBCAR269A, its wholly-owned investigational allogeneic CAR T candidate targeting B-cell maturation antigen for the treatment of R/R multiple myeloma, for which Precision has received Fast Track Designation and Orphan Drug Designation from the FDA. In February 2021, the study began enrolling patients into its highest dose cohort, dose level 3 (6.0 × $10^6$ cells/kg) and Precision expects to report interim data in 2021.

Precision also expects to initiate, in the first half of 2021, the combination arm of its ongoing Phase 1/2a clinical study with PBCAR269A and nirogacestat, SpringWorks Therapeutics' investigational gamma secretase inhibitor. Precision announced its clinical collaboration with SpringWorks in 2020.

***In Vivo* Gene Correction Portfolio:**

**Established Genome Editing Research Collaboration with Eli Lilly:** In November 2020, Precision and Eli Lilly and Company announced a research collaboration and exclusive license agreement to use Precision's proprietary ARCUS genome editing platform for the research and development of potential *in vivo* therapies for genetic disorders. The agreement includes up to six programs, with an initial focus on Duchenne muscular dystrophy and two other undisclosed gene targets.

In connection with the closing of the agreement in January 2021, Precision received an upfront cash payment of $100 million and Eli Lilly made an equity investment of $35 million in Precision's common stock. Precision is also eligible to receive up to an aggregate of $420 million in potential development and commercialization milestone payments per licensed product, nominating fees for additional targets, and tiered royalties ranging from the mid-single digits to low-teens on product sales should Lilly successfully commercialize a therapy from the collaboration.

**PH1:** Pre-clinical research continues to progress with Precision's wholly-owned *in vivo* gene correction program using its ARCUS genome editing technology to knock out the *HAO1* gene as a potential one-time treatment for primary hyperoxaluria type 1 (PH1), a rare genetic disease. The Company expects to provide an update on this program in the first half of 2021.

**PCSK9:** In February 2021, *Molecular Therapy* published a paper describing three-year follow-up data showing long-term stable reduction of low-density lipoprotein (LDL) cholesterol levels in nonhuman primates (NHPs) following *in vivo* gene editing of the PCSK9 gene with ARCUS genome editing. The study was led by James M. Wilson, M.D., Ph.D. and Lili Wang, Ph.D. from the Gene Therapy Program in the Perelman School of Medicine at the University of Pennsylvania. After a one-time vector administration in 2017, NHPs treated with ARCUS have experienced stable reductions of up to 85% in PCSK9 protein levels and a 56% reduction of LDL cholesterol levels.

**Corporate:**

**Intellectual Property Protection:** In January 2021, Precision received a Notice of Allowance from the U.S. Patent and Trademark Office for a patent application covering PBCAR19B. The allowed composition claims of this patent application encompass genetically-modified human T cells comprising the Company's PBCAR19B construct, which is inserted within the T cell receptor alpha constant locus. Once issued, patents arising from this patent family will have standard expiration dates in April 2040.

**Leadership:** In December 2020, Precision announced that Alex Kelly, the Company's Chief Corporate Affairs Officer, would serve as the Company's Interim Chief Financial Officer and Shane Barton, the Company's Vice President and Corporate Controller, would serve as interim principal accounting officer. These leadership changes followed the departure of Abid Ansari, Precision's prior Chief Financial Officer, after nearly five years with the organization to pursue a new career opportunity.

**Elo Life Systems:**

**Corporate Structure:** In January 2021, Precision disclosed its intention to spin out its wholly-owned subsidiary, Elo Life Systems. Precision is continuing to explore its strategic options, and the timing of any such sale, spinout or other treatment of Elo remains uncertain.

**Published Vanilla Genome Paper in *Nature Food*:** In December 2020, researchers at Elo Life Systems in collaboration with Alan Chambers, Ph.D., and the Tropical Research and Education Center at the University of Florida published a paper in *Nature Food*, reporting on a chromosome-scale, phased *Vanilla planifolia* genome, which revealed sequence variants for genes that may impact the vanillin pathway, and therefore influence bean quality, including its productivity, flower anatomy, and disease resistance.

**Fiscal Year 2020 Financial Results**

**Cash and Cash Equivalents:** As of December 31, 2020, Precision had approximately $89.8 million in cash and cash equivalents, which did not include the $100 million upfront cash payment and $35 million equity investment received from Eli Lilly in connection with the closing of the collaboration and license agreement in January 2021. The Company expects that cash and cash equivalents as of December 31, 2020, cash payments received from Lilly in January 2021, expected operational receipts and available credit will allow the Company to continue its operations into 2023.

**Revenues:** Total revenues for the year ended December 31, 2020 were $24.3 million, compared to $22.2 million for the year ended December 31, 2019. The increase of $2.1 million in revenue was primarily the result of an increase in collaboration revenue with Servier, offset by a decrease in collaboration revenue from Gilead due to the termination our agreement with them.

**Research and Development Expenses:** Research and development expenses were $98.1 million for the year ended December 31, 2020, as compared to $82.4 million for the same period in 2019. The increase of $15.7 million was primarily due to increases in direct research and development expenses related to our ongoing CD19 clinical program, increases in employee-related costs associated with increased headcount to support our technology platform development and manufacturing capabilities, and increased costs associated with contract manufacturing organizations and research organizations.

**General and Administrative Expenses:** General and administrative expenses were $36.1 million for the year ended December 31, 2020, as compared to $27.0 million for the same period in 2019. The increase of $9.1 million was primarily due to increases in employee-related costs associated with increased headcount as well as costs associated with the Company's growing infrastructure needs.

**Net Loss:** Net loss was $109.0 million, or $(2.09) per share, for the year ended December 31, 2020, compared to a net loss of $92.9 million, or $(2.21) per share, for the same period in 2019.

**About Precision BioSciences, Inc.**
Precision BioSciences, Inc. is a clinical stage biotechnology company dedicated to improving life (DTIL) with its wholly 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 correction therapy candidates to cure genetic and infectious diseases where no adequate treatments exist. For more information about Precision BioSciences, please visit www.precisionbiosciences.com.

**About Precision's Collaboration with Servier**
Under the terms of the agreement with Servier, Precision is solely responsible for early-stage research activities as well as PBCAR0191 Phase 1/2a and PBCAR19B Phase 1 clinical trial execution and clinical supply. Servier has the exclusive right to opt in for late-stage development and commercialization, and Precision has the right to participate in the development and commercialization of any licensed products resulting from the collaboration through a 50/50 co-development and co-promotion option in the United States.

**About Precision's Collaboration with Eli Lilly**
Under the terms of the research collaboration and exclusive license agreement with Eli Lilly, Precision will use its ARCUS genome editing platform for pre-clinical research and IND-enabling activities for up to six *in vivo* gene editing programs, with an initial focus on focus on Duchenne muscular dystrophy and two other undisclosed gene targets. Lilly will assume responsibility for clinical development and commercialization and will have the right to select up to three additional gene targets for this collaboration. Precision can co-fund clinical development of one product in exchange for an increased royalty rate on co-funded product sales.

**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 expected timing of trials and results from clinical studies of our CAR T product candidates and our *in vivo* gene correction program; the efficacy of our ARCUS genome editing technology; expected benefits of and payment pursuant to the collaboration with Eli Lilly; 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," "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 September 30, 2020, 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, 2020, accessible on the SEC's website at www.sec.gov and the Investors & Media page of our website 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.

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**Precision Biosciences, Inc.**
**Consolidated Statements of Operations**
(In thousands, except share and per share amounts)

| | For the Years Ended December 31, | |
|---|---|---|
| | **2020** | **2019** |
| Revenue | $ 24,285 | $ 22,238 |
| Operating expenses | | |
| Research and development | 98,061 | 82,416 |
| General and administrative | 36,052 | 27,026 |
| Total operating expenses | 134,113 | 109,442 |
| Loss from operations | (109,828) | (87,204) |
| Other income (expense), net: | | |
| Change in fair value of convertible note payable | — | (9,758) |
| Interest expense | — | (182) |
| Interest income | 822 | 4,267 |
| Total other income (expense), net | 822 | (5,673) |
| Net loss and net loss attributable to common stockholders | $ (109,006) | $ (92,877) |
| Net loss per share attributable to common stockholders-basic and diluted | $ (2.09) | $ (2.21) |
| Weighted average shares of common stock outstanding-basic and diluted | 52,031,740 | 41,991,162 |

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

| | **December 31, 2020** | **December 31, 2019** |
|---|---|---|
| Cash and cash equivalents | $ 89,798 | $ 180,886 |
| Working capital | 62,735 | 166,740 |
| Total assets | 150,158 | 235,233 |
| Total stockholders' equity | $ 44,425 | $ 138,314 |