



Precision BioSciences Recaps 2022 Accomplishments and Outlines 2023 Corporate Priorities and Planned Portfolio Milestones

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DURHAM, N.C.--(BUSINESS WIRE)--Jan. 9, 2023-- Precision BioSciences (Nasdaq: DTIL), a clinical stage gene editing company developing ARCUS®-based *ex vivo* allogeneic CAR T and *in vivo* gene editing therapies, today reported on its significant accomplishments in 2022 and announced its corporate priorities and anticipated clinical development and research milestones for 2023.

"In 2022, we advanced our corporate priorities strengthening the company and made significant progress with our clinical programs and research pipeline. We established a new, premium *in vivo* gene editing collaboration with Novartis focused on gene insertion for sickle cell disease and beta thalassemia, and we added \$125 million to our balance sheet to fortify the company and extend our cash runway. We also published the first non-human primate proof of concept data supporting further development of our HBV *in vivo* gene editing program and made significant progress on our partnered programs with Lilly and Novartis involving complex gene edits such as excision and gene insertion," said Michael Amoroso, President and Chief Executive Officer of Precision BioSciences.

"We made meaningful clinical, manufacturing and regulatory progress on our allogeneic CAR T programs. From a clinical perspective, in the latest cohort of our Phase 1b clinical study reported in mid-2022, azercabtagene zapreleucel (azer-cel; PBCAR0191) achieved a 100% overall response rate and 73% complete response to treatment.¹ In addition, we implemented process optimizations for our allogeneic CAR T manufacturing process, significantly improving product attributes that we believe are correlated with improved efficacy and safety, and concluded the year with favorable FDA feedback signaling alignment with our proposed CMC plan for azer-cel, our lead clinical candidate."

Today, Precision announced its key priorities and planned upcoming milestones for 2023, including:

1. Progress azer-cel to decision point for Phase 2 trial in non-Hodgkin lymphoma (NHL) subjects who have relapsed following autologous CAR T treatment.

- Complete Phase 1b cohort for azer-cel to determine final dosing schedule; request FDA clinical meeting pending data.
- **Complete Phase 1 dose escalation for PBCAR19B** in the earlier line NHL setting.
- Present CAR T clinical update in the first quarter of 2023, based on patient accrual and follow-up.

2. Advance wholly owned PBGENE-HBV *in vivo* program to final clinical candidate enabling target CTA and/or IND filing in 2024.

- Data published in 2022 demonstrates that ARCUS efficiently targeted and degraded hepatitis B virus (HBV) cccDNA by 85% and durably reduced expression of HBV S-antigen by 77% in HBV-infected primary human hepatocytes (PHH). Importantly, optimized specificity of the ARCUS nuclease completely prevented detectable chromosomal translocations in the PHH model.
- Using **lipid nanoparticle (LNP) delivery**, ARCUS nucleases showed high on-target editing and a robust decrease in viral DNA in both mouse and non-human primate models, along with 96% sustained reduction of HBV S-antigen in mice.
- The Company plans to present additional data at a scientific conference in 2023.

3. Advance first ARCUS *in vivo* gene editing program to clinical readiness.

- In partnership with iECURE, an ARCUS-mediated **gene insertion** approach is being pursued as a potential treatment for neonatal onset ornithine transcarbamylase (OTC) deficiency.
- Non-human primate 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 non-human primates with high efficiency.
- iECURE targeting to file CTA and/or IND in second half of 2023.

4. Progress key partnered programs toward IND, including Duchenne muscular dystrophy and sickle cell disease.

- Through partnerships with Novartis and Lilly, the versatility of ARCUS is highlighted for complex editing and gene insertion in diverse tissues, including muscle and hematopoietic stem cells.
- Joint teams continue to make significant progress against preclinical objectives.

5. Extend cash runway.

- Although it has not finalized its full financial results, Precision expects to report it had approximately \$190 million in cash and cash equivalents as of December 31, 2022. The Company's management also believes that, as of January 9, 2023, the Company's existing cash and cash equivalents, expected operational receipts, and available credit will be sufficient to fund its operating expenses and capital expenditure requirements into 2025.

In Vivo Gene Editing Strategy Update:

The strategic prioritization exercise for Precision's *in vivo* research pipeline, announced during the Company's third quarter earnings, is ongoing to assess diseases with highest unmet need in an increasingly dynamic regulatory and competitive gene editing landscape.

"The Precision team, along with its partners, have generated an abundance of preclinical data in 2022 and had significant learnings," said Derek Jantz, Ph.D., Chief Scientific Officer. "The Precision team along with partners continue to validate unique features of the ARCUS platform with regards to safety, on-target editing, gene insertion, complex gene edits, and compatibility with viral and non-viral delivery. We remain very excited about Precision's proprietary and foundational ARCUS platform and are steadfast about moving the first ARCUS *in vivo* gene editing program into the clinic as rapidly as possible."

"Our HBV program remains a top priority, and we are on track with our original goal of submitting a CTA and/or IND in 2024. We look forward to providing periodic updates on the team's progress. In addition, in our partner's hands at iECURE, operational progress has been made on a first gene insertion program and the CTA filing for neonatal onset OTC deficiency is planned for submission this year," said Dr. Jantz.

As a result of the ongoing prioritization, the Company is making trade-offs and further honing its focus on disease areas where the Company believes ARCUS, more than any other technology, can have the greatest and most profound impact. While the Company will continue to pursue gene knock-out programs opportunistically, the proof of concept data continues to lead toward prioritizing programs involving complex edits, as with the HBV and DMD programs, and gene insertion (adding a functional copy of a gene) as exemplified by our partnered OTC program.

While Precision remains committed to patients with cardiovascular diseases, it has made the decision to cease pursuit of PBGENE-PCSK9 for familial hypercholesterolemia (FH) with iECURE as its partner. PCSK9 for FH remains a wholly-owned program, and the Company is monitoring the regulatory landscape as it considers FH as well as several potential cardiovascular disease indications in its pipeline prioritization exercise. A more robust plan for development of Precision's *in vivo* pipeline, including research milestones, will be outlined at an R&D Day in mid-2023.

Finally, work on the PBGENE-PH1 program progressed as planned in 2022. Precision has clinical candidates ready to proceed to the next stage of IND enabling studies. Based on Precision's new prioritized focus as well as the evolving treatment paradigm for PH1, the Company has made the choice to look for a partner in the kidney disease arena for further development of PBGENE-PH1 and will no longer develop the program on its own.

"Precision has significant optionality with its *in vivo* gene editing pipeline and we look forward to discussing more about our prioritization, learnings with ARCUS, and next steps at the 2023 R&D Day," said Mr. Amoroso.

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. The Company intends such forward-looking statements to be covered by the safe harbor provisions for forward-looking statements contained in Section 27A of the Securities Act and Section 21E of the Exchange Act. 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 research advancement, clinical development and regulatory review of our product candidates, the expected timing of updates regarding our CAR T and *in vivo* gene editing programs, the expected timing of our communications with regulators, expected efficacy and benefit of our product candidates and programs, expectations about our operational initiatives and our business strategy, achieving key milestones and additional collaborations, and expectations regarding our cash balance and ability to fund operating expenses and capital expenditure requirements. The words "aim," "anticipate," "approach," "believe," "contemplate," "could," "estimate," "expect," "goal," "intend," "look," "may," "mission," "plan," "possible," "potential," "predict," "project," "promise," "pursue," "should," "target," "will," "would," and other similar words or expressions, or the negative of these words or similar words or expressions, are intended to identify forward-looking statements, though not all forward-looking statements use these words or 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, but involve 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 risk that other genome-editing technologies may provide significant advantages over 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 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' 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; effects of sustained inflation, supply chain disruptions and major central bank policy actions; 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, 2022, 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.

Financial Disclosure Advisory

The 2022 financial results included in this press release are unaudited and preliminary, and this press release does not present all information necessary for an understanding of the Company's financial condition as of December 31, 2022 and its results of operations for the three months and year ended December 31, 2022. The Company's actual results may differ from the preliminary estimates above due to the completion of the Company's year-end accounting procedures, including execution of the Company's internal control over financial reporting, and audit of the Company's financial statements for the year ended December 31, 2022 by the Company's independent registered public accounting firm, which are ongoing.

¹ Results announced on June 8, 2022 (n=12) were as of May 31, 2022. 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.

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