



## Precision BioSciences Announces the Presentation of Initial Clinical Data Supporting the Safety and Clinical Activity of PBCAR0191, a Novel CD19 Targeted Allogeneic CAR T Therapy Candidate, at the American Society of Hematology Annual Meeting

November 6, 2019

*-Preliminary data from ongoing Phase 1/2a trial of PBCAR0191 for the treatment of relapsed/refractory non-Hodgkin lymphoma or relapsed/refractory B-cell precursor acute lymphoblastic leukemia-*

*-Abstract summarizes early results from first three patients with advanced NHL treated with PBCAR0191 at Dose Level 1 as of August 1, 2019-*

*-No serious adverse events or dose-limiting toxicities observed over median 60 days follow-up. Two of three patients experienced objective tumor responses by Lugano criteria, with the third patient having evidence of anti-tumor activity-*

*-Trial is ongoing and updated data, including from patients treated at Dose Level 2, will be presented during the ASH Annual Meeting-*

*-Precision BioSciences to host investigator update event during ASH to discuss these data, starting at 8:15 p.m. ET on December 9, with live webcast available online-*

DURHAM, N.C., Nov. 06, 2019 (GLOBE NEWSWIRE) -- Precision BioSciences, Inc. (Nasdaq: DTIL), a genome editing company dedicated to improving life through the application of its pioneering, proprietary ARCUS® platform, announced today that initial results from the ongoing Phase 1/2a trial of its lead investigational off-the-shelf (allogeneic) chimeric antigen receptor (CAR) T cell therapy candidate, PBCAR0191, will be presented during the 61<sup>st</sup> Annual Meeting of the American Society of Hematology (ASH) in Orlando, Florida, December 7-10, 2019.

PBCAR0191 is Precision's first allogeneic CAR T therapy candidate in clinical trials and targets the well characterized cancer cell surface protein CD19. It is being developed in collaboration with Servier, an international pharmaceutical company. The Phase 1/2a trial includes adult patients with relapsed or refractory (R/R) non-Hodgkin lymphoma (NHL) or R/R B-cell precursor acute lymphoblastic leukemia (B-ALL). The abstract outlining initial data from patients treated with PBCAR0191 at Dose Level 1 is available on the ASH conference website (<https://www.hematology.org/Annual-Meeting/Abstracts>). This trial is ongoing and updated results, including from patients treated at Dose Level 2, will be presented at the ASH Annual Meeting on December 9, 2019 starting at 6:00 p.m. ET.

"We are excited to share initial clinical data from Precision's PBCAR0191 program at ASH, which we believe demonstrate the potential of our differentiated approach to the development of allogeneic CAR T therapies," said Chris Heery, MD, Chief Medical Officer of Precision BioSciences. "These data bring the reality of a true off-the-shelf CAR T therapy a step closer for patients in need of new and improved treatment options. We remain committed to the wider goal of improving access to cellular therapies for patients with advanced NHL and ALL, and we are optimistic, based on these initial findings, that we may be able to help meet this need. While preliminary and from a limited number of patients, the safety profile, *in vivo* cell expansion and early evidence of clinical activity we have demonstrated at our lowest dose level with PBCAR0191 in the absence of biologic lymphodepletion is very encouraging. We look forward to sharing updated results from patients treated at Dose Levels 1 and 2 at ASH."

### Investigator Update & Webcast Information

Precision will host a live webcast of an investigator update event during the ASH Annual Meeting to discuss the presented data, beginning at 8:15 p.m. ET on Monday, December 9, 2019. To access the webcast, please visit the "Events & Presentations" page within the Investors & Media section of the Precision BioSciences website at <http://investor.precisionbiosciences.com/>. A replay of the webcast will be available on the Precision website for 30 days following the call.

### First Clinical Data from Precision's PBCAR0191 Program

**Title:** Initial findings of the Phase 1 trial of PBCAR0191

**Presenter:** Bijal Shah, MD, Moffitt Cancer Center

**Session:** 627. Aggressive Lymphoma (Diffuse Large B-Cell and Other Aggressive B-Cell Non-Hodgkin Lymphomas)—Results from Retrospective/Observational Studies

**Poster/Presentation Number:** Poster III

**Date and Time:** December 9, 2019, 6:00-8:00 p.m. ET

**Location:** Orange County Convention Center, Hall B

PBCAR0191 is Precision's first allogeneic CAR T therapy candidate in clinical trials. This presentation will include initial data from the Phase 1 portion of the ongoing Phase 1/2a trial of PBCAR0191, which is designed to assess safety, identify an optimal dose of PBCAR0191 and evaluate preliminary clinical activity in patients with R/R NHL and B-ALL. The trial is a 3+3 dose escalation study (at dose levels of  $3 \times 10^5$ ,  $1 \times 10^6$ , and  $3 \times 10^6$  CAR T+ cells/kg); in each of the three dose levels up to six patients may be enrolled in each of the two cohorts (NHL and B-ALL). Lymphodepletion is achieved using fludarabine  $30 \text{mg/m}^2/\text{day}$  and cyclophosphamide  $500 \text{mg/m}^2/\text{day}$ .

Data in the abstract include results as of the data cutoff date of August 1, 2019 for three patients with advanced NHL treated at Dose Level 1, one with mantle cell lymphoma (MCL) and two with diffuse large B cell lymphoma (DLBCL). No significant toxicity was observed, including no serious adverse

events and no dose-limiting toxicities. All patients had a minimum follow-up of 28 days (median 60 days).

Findings indicate preliminary evidence of cell-mediated anti-tumor activity, which will be evaluated more fully at subsequent dose levels. Two of the three patients experienced an objective tumor response by Lugano criteria, at day 14 and day 28, respectively. Both patients progressed due to new lesions (on day 28 and day 60, respectively). The third patient, who had previously progressed following treatment with axicabtagene ciloleucel (Yescarta®), an approved anti-CD19 autologous CAR T therapy, had not met the definition of response, but had shown evidence of central necrosis, decreased tumor size, and decreased PET-avidity at day 28, in the context of post-infusion tumor site pain and mild CRS symptoms.

Peripheral blood analysis for CAR T cell expansion has identified preliminary evidence of cell expansion.

This trial is ongoing and updated results, including from patients treated at Dose Level 2, will be shared at the ASH conference.

### **Precision's Off-The-Shelf CAR T Platform**

Precision is advancing a pipeline of cell-phenotype optimized allogeneic CAR T therapies, leveraging fully scaled, proprietary manufacturing processes. The platform is designed to maximize the number of patients who can potentially benefit from CAR T therapy by improving access to care through a well-tolerated lymphodepletion regimen. Precision carefully selects high-quality T cells derived from healthy donors as starting material, then utilizes its unique ARCUS genome editing technology to modify the cells via a single-step engineering process. By inserting the CAR gene at the T cell receptor (TCR) locus, this process knocks in the CAR while knocking out the TCR, creating a consistent product that can be reliably and rapidly manufactured and is designed to prevent graft-versus-host disease. Precision optimizes its CAR T therapy candidates for immune cell expansion in the body by maintaining a high proportion of naïve and central memory CAR T cells throughout the manufacturing process and in the final product.

### **About Precision BioSciences, Inc.**

Precision BioSciences is dedicated to improving life (DTIL) through its proprietary genome editing platform, ARCUS. Precision leverages ARCUS in the development of its product candidates, which are designed to treat human diseases and create healthy and sustainable food and agriculture solutions. Precision is actively developing product candidates in three innovative areas: allogeneic CAR T immunotherapy, *in vivo* gene correction, and food. For more information regarding Precision, please visit [www.precisionbiosciences.com](http://www.precisionbiosciences.com).

### **About Precision's Collaboration with Servier**

Under the February 2016 partnership with Baxalta, now with Servier, Precision is solely responsible for early-stage research activities and Phase 1 execution for PBCAR0191, as well as preparation of clinical supply for any Phase 2 clinical trials. 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.

### **Forward-Looking Statements**

Information contained in or accessible through this press release contains forward-looking statements. All statements other than statements of present and historical facts contained in or through this press release, including without limitation, statements regarding the potential for PBCAR0191 to provide a therapy option for patients living with NHL or B-ALL. In some cases, you can identify forward-looking statements by terms such as "anticipate," "believe," "could," "expect," "should," "plan," "intend," "estimate," "target," "mission," "may," "will," "would," "should," "could," "target," "project," "predict," "contemplate," "potential," or the negative thereof and similar words and expressions.

Forward-looking statements are based on management's 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 factors, including, but not limited to, our ability to become profitable; our ability to procure sufficient funding; our limited operating history; our ability to identify, develop and commercialize our product candidates; our dependence on our ARCUS technology; the initiation, cost, timing, progress and results of research and development activities, preclinical or greenhouse studies and clinical or field trials; our or our collaborators' ability to identify, develop and commercialize product candidates; our or our collaborators' ability to advance product candidates into, and successfully complete, clinical or field trials; our or our collaborators' ability to obtain and maintain regulatory approval of future product candidates, and any related restrictions, limitations and/or warnings in the label of an approved product candidate; the regulatory landscape that will apply to our and our collaborators' development of product candidates; our ability to achieve our anticipated operating efficiencies as we commence manufacturing operations at our new facility; our ability to obtain and maintain intellectual property protection for our technology and any of our product candidates; the potential for off-target editing or other adverse events, undesirable side effects or unexpected characteristics associated with any of our product candidates; the success of our existing collaboration agreements; our ability to enter into new collaboration arrangements; public perception about genome editing technology and its applications; competition in the genome editing, biopharmaceutical, biotechnology and agricultural biotechnology fields; potential manufacturing problems associated with any of our product candidates; potential liability lawsuits and penalties related to our technology, our product candidates and our current and future relationships with third parties; 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, 2019, as 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](http://www.sec.gov).

All forward-looking statements speak only as of the date of this press release and, except as required by applicable law, we do not plan to publicly 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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