



Precision BioSciences Presents Updated Interim Clinical Data at the ASH Annual Meeting from Relapsed / Refractory NHL and B-ALL Patients Treated at Dose Levels 1 & 2 in the Ongoing Phase 1/2a Clinical Trial of PBCAR0191, a Novel CD19 Targeted Allogeneic CAR T Therapy Candidate

December 9, 2019

-PBCAR0191 continues to be associated with no serious adverse events or dose limiting toxicities observed to date, and no cases of graft-versus-host disease-

-Seven of nine treated patients (78%) had objective evidence of tumor shrinkage at any timepoint-

-R/R non-Hodgkin lymphoma (NHL) cohort achieved day 28+ objective response rate (ORR) of 66% (4/6 patients across DL1 and DL2) including one complete response (CR) and three partial responses (PRs); two further patients achieved early responses that progressed. R/R B-cell precursor acute lymphoblastic leukemia (B-ALL) cohort achieved day 28+ ORR of 33% (1/3 patients at DL2) comprising one CR – two patients who did not respond had poor prognosis at trial entry-

-Progression free survival up to 180 days in NHL cohort – most durable response so far seen is in one NHL patient who previously relapsed following treatment with Yescarta®-

-Preliminary exploratory evidence of dose-dependent CAR T cell expansion and persistence. B-cell aplasia observed in all patients corresponding to timing of expected peak cell expansion and persistence-

-Trial is ongoing and updated data, including from patients treated at Dose Level 3, expected to be presented in Q1 2020-

-Precision BioSciences to host investigator update event today starting at 8:15 p.m. ET, with live webcast available online-

DURHAM, N.C., Dec. 09, 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, today announced updated interim clinical data from the ongoing Phase 1 trial of its lead investigational off-the-shelf (allogeneic) chimeric antigen receptor (CAR) T cell therapy candidate, PBCAR0191, which targets the well characterized cancer cell surface protein CD19. PBCAR0191 is being developed in collaboration with Servier, an international pharmaceutical company. Data will be presented by Bijal Shah, MD, Moffitt Cancer Center, at the 61st Annual Meeting of the American Society of Hematology (ASH) in Orlando, Florida, during a poster session from 6:00-8:00 p.m. ET today (Poster #4107, Hall B).

"We are very encouraged by the evidence of cell-mediated anti-tumor activity and objective tumor responses that we have observed in both NHL and B-ALL patients treated with PBCAR0191, in the context of a manageable adverse event profile," said Chris Heery, MD, Chief Medical Officer of Precision BioSciences. "These data give us incremental confidence in our unique approach to allogeneic CAR T cell therapy, and we look forward to the potential of this therapy positively impacting the lives of more patients as the trial continues. At these still low dose levels, and using only mild lymphodepletion, it is remarkable to see anti-tumor activity in the majority of patients treated with PBCAR0191, including a durable response that lasted six months in one patient, and two complete responses. We have also seen preliminary evidence of dose dependent CAR T cell expansion and persistence, supporting our belief that cell persistence and clinical response is likely to increase as we increase dose level."

"New treatment options are desperately needed for patients with advanced NHL and B-ALL who often undergo multiple cycles of therapy with limited clinical benefit," commented Bijal Shah, MD, Moffitt Cancer Center. "These first-in-human data for PBCAR0191 suggest a tolerable safety profile and encouraging early evidence of clinical activity. Further study is required to determine durability of response at the current dose levels, as well as to establish safety and activity at Dose Level 3."

Patient baseline characteristics and trial overview

A total of nine patients are reported in these initial Phase 1 trial results, including six with NHL (three treated at Dose Level 1 and three treated at Dose Level 2), and three with B-ALL (all treated at Dose Level 2). Key baseline characteristics were as follows:

- **Dose Level 1** (3×10^5 cells/kg) – three NHL patients (two with diffuse large B cell lymphoma, one with mantle cell lymphoma) with a mean age of 54 years (min-max 34-64 years). Patients had received a median of four prior lines of therapy, with two patients being refractory to their last treatment, and one having previously relapsed following treatment with Yescarta®, an FDA-approved autologous CD19-targeted CAR T therapy.
- **Dose Level 2** (1×10^6 cells/kg) – three NHL patients (all with mantle cell lymphoma) with a mean age of 74 years (min-max 71-77 years) who had received a median of two prior lines of therapy, with one patient refractory to their last treatment and two who had relapsed. Three B-ALL patients were also treated at DL2, with a mean age of 56 years (min-max 48-72 years); these patients had received a median of four prior lines of therapy – all three patients were refractory to their last treatment, with two patients having poor prognostic indicators at trial entry.

Patients received a single infusion of PBCAR0191 on day 0, following three days of lymphodepletion using fludarabine 30mg/m²/day and cyclophosphamide 500mg/m²/day. The primary objective of this Phase 1 portion of the ongoing Phase 1/2a trial is to evaluate safety as measured by the occurrence of dose limiting toxicities (DLTs). Secondary objectives include assessment of objective tumor responses using standard criteria, and further evaluation of adverse events (AEs) and adverse events of special interest, including graft-versus-host disease (GvHD), cytokine release syndrome (CRS), and IEC-associated neurotoxicity syndrome (ICANS). Data are presented as of a November 4, 2019 cutoff date, with additional critical data collected through December 2, 2019, including occurrence of CRS, ICANS, GvHD and evaluation of objective responses.

Safety of PBCAR0191

No serious adverse events or evidence of GvHD was observed through December 2, 2019. Three of the nine patients (33%) treated with PBCAR0191 developed CRS, including two Grade 1 cases and one Grade 2 case. One of the nine patients (11%) developed Grade 2 neurotoxicity. All events of CRS and neurotoxicity resolved, and no deaths occurred on study. In addition, one patient experienced a Grade 3 AE that was deemed related to PBCAR0191 (pain at the site of their tumor mass for one day following infusion), and one patient experienced Grade 4 lymphopenia (seven days duration) deemed related to PBCAR0191.

Clinical activity of PBCAR0191

Of the nine patients treated with PBCAR0191, seven (78%) had objective evidence of tumor shrinkage at any timepoint. Results also provide preliminary evidence of dose-dependent CAR T cell expansion and persistence.

In the NHL cohort, four of six patients (66%) achieved an objective response by Lugano 2014 criteria at day 28+, including three partial responses (two patients treated at DL1 and one patient treated at DL2) and one complete response (patient treated at DL2). As of December 2, 2019, one patient (treated at DL2) remains in complete response. One patient (treated at DL1) achieved a partial response then progressed six months after treatment with PBCAR0191. This was the most durable response observed to date and was also notable given the patient had relapsed following treatment with Yescarta®. The remaining two NHL patients, one treated at DL1 and one at DL2, achieved early responses (one CR, one PR respectively) at day 14; both patients had evidence of disease progression at day 28.

In the B-ALL cohort treated at DL2, one of three patients (33%) achieved a complete response by NCCN 2017 criteria at day 28+ (with undetectable B-ALL in the bone marrow by flow cytometry, described as minimal residual disease (MRD) negative), and continues to be followed on study. The remaining two patients did not respond at day 28 – these patients had poor prognostic indicators on entry into the trial, one with prior CNS involvement and 95% blast infiltration into the bone marrow, and one with 77% blast infiltration into the bone marrow and disease refractory to two previous lines of treatment.

CAR T cell expansion and persistence in the peripheral blood was assessed at DL1 and DL2 by flow cytometry and qPCR. Evidence of a dose-dependent increase in cell expansion was observed between subjects treated at DL1 and DL2, as was a dose-dependent increase in CAR T cell persistence. B-cell aplasia and serum cytokine analysis also anecdotally correspond to observed clinical responses and CAR T cell expansion.

This trial is ongoing and treatment of patients at DL3 (3x10⁶ cells/kg) recently commenced. Updated results, including from patients treated at DL3, are expected to be presented at a medical meeting in the first quarter of 2020.

Investigator Update & Webcast Information

Precision will host a live webcast of an investigator update event during the ASH Annual Meeting to discuss these 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 event.

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, high quality cell products derived from carefully selected healthy donors, and a consistent final cell product with attributes in line with those previously observed to result in optimal safety and activity profiles. 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 in a single step, 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 naive 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.

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 this press release contains forward-looking statements. All statements other than statements of present and historical facts contained in this press release, including without limitation, statements regarding the potential for the successful development of PBCAR0191 for patients living with NHL or B-ALL, may be forward looking statements. Without limiting the foregoing, the words "anticipate," "believe," "could," "expect," "should," "plan," "intend," "estimate," "target," "may," "will," "would," "potential," the negative thereof and similar words and expressions are intended to identify forward-looking statements. These forward-looking statements reflect various assumptions of Precision's management that may or may not prove to be correct. No forward-looking statement is a guarantee of future results, performance, or achievements, and one should avoid

placing undue reliance on such statements.

Forward-looking statements are based on our 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 filed for the quarterly period ended September 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.

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.

Investor Contact:

Nick Riddle
Precision BioSciences
Tel. (919) 314-5512
IR@precisionbiosciences.com

Media Contact:

Cory Tromblee
Scient Public Relations
Tel. (617) 571-7220
cory@scientpr.com