Precision BioSciences Announces FDA Clearance of the IND for PBCAR20A, a CD20 Targeting Genome Edited Allogeneic CAR T Therapy

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- *Phase 1/2a Clinical Trial of Off-The-Shelf Anti-CD20 CAR T Therapy Candidate in Patients with NHL, CLL, and SLL Expected to Begin in Fourth Quarter 2019*
- *PBCAR20A has Received Orphan Drug Designation for Mantle Cell Lymphoma*

DURHAM, N.C., Sept. 16, 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 the U.S. Food and Drug Administration (FDA) has accepted its Investigational New Drug (IND) application for PBCAR20A, the Company's second off-the-shelf chimeric antigen receptor (CAR) T cell therapy program. Wholly owned by Precision, PBCAR20A is an allogeneic anti-CD20 CAR T therapy candidate in development for the treatment of non-Hodgkin lymphoma (NHL), chronic lymphocytic leukemia (CLL), and small lymphocytic lymphoma (SLL). The company plans to initiate a Phase 1/2a clinical trial in the fourth quarter of 2019, with initial data expected in 2020. The study will include patients with NHL, of which a subset will have the diagnosis of mantle cell lymphoma (MCL). Precision BioSciences has received Orphan Drug Designation for MCL and plans to pursue this indication.

"FDA clearance to begin clinical trials with our anti-CD20 off-the-shelf CAR T therapy candidate is a significant milestone for Precision. Closely following the initiation earlier this year of our first clinical trial with the anti-CD19 allogeneic CAR T therapy candidate PBCAR0191, today's announcement demonstrates our ability to advance multiple product candidates in parallel into the clinic, leveraging the unique capabilities of our ARCUS genome editing platform, CAR T development approach and a highly differentiated manufacturing process developed in house," commented Matt Kane, Chief Executive Officer and Co-Founder of Precision BioSciences. "I am delighted that our team continues to deliver ahead of expectations."

David Thomson, Chief Development Officer of Precision, said, "In preclinical disease models, PBCAR20A has demonstrated potent *in vivo* clearance of CD20+ tumor cells and overall tumor volume reduction. Furthermore, we did not observe any evidence of graft-versus-host disease in strict preclinical models. It is our hope that PBCAR20A will provide

a new allogeneic CAR T therapy option with the benefits of reliable, off-the-shelf access and optimized cellular activity to patients living with NHL or CLL/SLL, where a significant need for new treatment options remains. Receiving orphan drug designation from the FDA for MCL means that we have a special opportunity to serve those patients previously treated for the MCL form of NHL, who have a poor prognosis with currently available treatments."

PBCAR20A will be Precision's second off-the-shelf CAR T therapy candidate to enter the clinic, following PBCAR0191, an off-the-shelf anti-CD19 CAR T therapy candidate currently being evaluated in adult patients with relapsed or refractory (R/R) NHL or R/R B-cell precursor acute lymphoblastic leukemia (B-ALL). Precision initiated dosing of subjects in a Phase 1/2a clinical trial of PBCAR0191 in April 2019, which continues to progress as planned; the company expects to present interim data from this trial at a scientific conference no later than the first quarter of 2020.

With the IND acceptance and expected upcoming clinical trial initiation for PBCAR20A, Precision's clinical-stage portfolio of off-the-shelf CAR T therapy candidates for B-cell malignancies continues to grow. In the United States, B-cell malignancies account for 85 percent of all NHL cases, and CLL and SLL represent 25 to 30 percent of leukemias. While front-line treatments provide benefit to more than half of newly diagnosed NHL patients, at least a third of those who do benefit will become refractory, achieve only partial remission or relapse after remission. In addition, patients with CLL have seen limited success with autologous CAR T cell therapies, which is commonly ascribed to T cell exhaustion associated with this malignancy. Administration of healthy donor T cells via allogeneic CAR T cell therapy has the potential to overcome current challenges in CLL treatment, increase access to care in NHL and overcome treatment resistance due to CD19 loss. In addition, allogeneic CAR T therapy presents the possibility for combination treatment options by targeting both CD19 and CD20, which may help overcome resistance mechanisms in some patients.

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. 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 the PBCAR20A Clinical Trial

PBCAR20A will be evaluated in a Phase 1/2a multicenter, nonrandomized, open-label, parallel assignment, single-dose, dose-escalation and dose-expansion clinical trial in adult NHL, CLL, and SLL patients. The trial will be conducted at multiple U.S. sites. For more information on the trial, visit **www.clinicaltrials.gov**, study identifier number NCT04030195.

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.**

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 statements regarding the Company’s plans to initiate the Phase 1/2a clinical trial for PBCAR20A in the fourth quarter of 2019, the potential for PBCAR20A to provide a therapy option for patients living with NHL or CLL/SLL, and the, expectation for additional CAR T candidates to enter clinical trials. 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 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; 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” 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.**

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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