PersImmune

UC San Diego
MOORES CANCER CENTER

Dear Dr.,

7/25/19

This letter is to inform you about a novel clinical trial for patients with myelodysplastic syndromes (MDS) that is sponsored by PersImmune, Inc. (IND #17310; NCT 03258359) and is currently available at UC San Diego Moores Cancer Center (with additional sites opening soon).

We are offering a phase 1 immunotherapy trial that will assess the safety of an adoptive T cell therapy (i.e., T cell infusion) for patients with **relapsed or refractory MDS or those who decline standard therapy**. This approach uses a patient's own T-cells to target their patient-specific neoantigens (cancer-associated peptides). These mutant proteins, derived from both cancer-driver mutations and passenger mutations are uniquely expressed by malignant cells and not by normal cells, which minimizes off-target toxicity of this therapy. Eligible subjects for the trial are patients who are refractory to or have relapsed following standard MDS therapy (i.e., hypomethylating agents) and are not eligible for hematopoietic stem cell transplantation. ***However, patients must first be enrolled in the tissue collection protocol (NCT 03072498), ideally prior to receiving MDS therapy.*** Then, if they refuse, relapse, or fail to respond, they can proceed to receive their experimental T-cell product through this clinical trial. Patients who were treated elsewhere and are refractory are also eligible if expected to live >6 months.

This trial is an open-label, non-randomized phase 1 study developed collaboratively by PersImmune, Inc., and Dr. Rafael Bejar at UC San Diego. The primary endpoints are to assess the safety, tolerability and maximum tolerated dose of the T cell therapeutic product. Secondary endpoints include assessment of disease response, overall and progression-free survival, and survival of the infused T cells.

We are currently recruiting patients. Notably, the T cell infusions have been very well tolerated by the first cohort of three patients treated under this protocol. Key information about referral and the study's main criteria is on the following page. To be eligible for the trial, patients need to have sufficient tissue (bone marrow, blood, and brief leukapheresis) collected on our IRB-approved protocol to send for genomic sequencing and to determine if targetable MDS neoantigens are present. ***Tissue collection will ideally occur prior to initiating standard therapy, thus an early referral at initial MDS diagnosis is needed. After the initial consultation and sample collection is completed at UC San Diego, the patient should return to your care until they are deemed refractory or relapsed on standard therapy. The study sponsor, PersImmune, will cover the financial cost of the initial office visit, tissue collection and the clinical trial. If a suitable T cell product is made, and once the patient is deemed refractory to standard therapy or relapsed, the patient would return to UCSD to be screened for the phase I trial prior to enrolling.***

Please feel free contact us for more information about this trial.

Sincerely,

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Chief Medical Officer,
PersImmune, Inc.

for

Rafael Bejar, MD PhD
Study PI
UCSD School of Medicine

and

Tiffany Tanaka, MD
Study Sub-Investigator
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STEP 1: Early Referral to UCSD for Tissue Collection (NCT 03072498) prior to treatment:

Noteworthy Inclusion Criteria

- Age ≥18 years of age
- Diagnosis of MDS by the French-American-British criteria
- Presumed to be higher risk MDS

STEP 2: Referral to UCSD for Clinical Trial (NCT 03258359) after patient is deemed refractory to treatment or relapses: Noteworthy Inclusion Criteria

- Age ≥18 years of age
- Confirmed diagnosis of MDS by the French-American-British criteria
- Intermediate, high or very high risk disease by the revised International Prognostic Scoring System for MDS
- Refractory disease or inadequate response to ≥6 cycles of hypomethylating agent therapy (azacitidine, decitabine) **OR** relapsed disease but participated in prior tissue collection protocol, **OR** declined hypomythylating therapy after initial diagnosis
- No MDS therapy for ≥28 days prior to receiving the investigational therapy
- Opted to forego or deemed ineligible for allogeneic hematopoietic stem cell transplantation

Noteworthy Exclusions for Clinical Trial include:

- ECOG status >2
- Prior history of allogeneic hematopoietic stem cell transplantation
- Residual adverse events from MDS therapy received ≥28 days later
- History of autoimmune disease
- Concurrent immunosuppressive therapy

Please contact our study team EARLY to refer patients you believe are eligible for this study. We will be happy to provide additional information about the study.

- Principal Investigator: Rafael Bejar, 858-534-5204, rabejar@ucsd.edu
- Sub-Investigator: Tiffany Tanaka, 858-534-5875, tntanaka@ucsd.edu
- Clinical Trials Manager: Kimberly Aguilar, 858-534-5201, k1aguilar@ucsd.edu
- Clinical Research Coordinator: Colin McCarthy, 858-534-8127, c4mccarthy@ucsd.edu

More information can be found at http://clinicaltrials.gov:

- Collection of Samples From Patients With MDS: NCT 03072498
- Personalized Adoptive Cellular Therapy Targeting MDS Stem Cell Neoantigens (PACTN): NCT 03258359

For additional questions re: the tissue collection protocol, clinical trial or the T cell product for infusion:

- Chief Medical Officer, PersImmune: Thomas Lane, 858 704-4499; tlane@persimmune.com

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