



Poseida Provides Update on Phase 1 Study of P-BCMA-101 CAR-T Stem Cell Memory Product in Patients with Relapsed/Refractory Multiple Myeloma

Efficacy and safety continue to be a major advantage, with a very high response rate, no dose limiting toxicities and only a single incidence of suspected cytokine release syndrome

P-BCMA-101 clinical data as well as preclinical data from MUC1C and PSMA solid tumor programs and BCMA allogeneic program presented at the CAR-TCR Summit

SAN DIEGO – September 5, 2018 – Poseida Therapeutics Inc. (“Poseida”), a clinical-stage biotechnology company translating best-in-class gene engineering technologies into lifesaving cell therapies, announced data results from the first eleven patients treated in its ongoing Phase 1 study of its P-BCMA-101 stem cell memory chimeric antigen receptor T-cell (CAR-T) product in relapsed/refractory multiple myeloma. All eleven patients remain on study with seven of ten patients evaluable by International Myeloma Working Group (IMWG) criteria achieving at least a partial response. The remaining patient also demonstrated a robust response, but has oligosecretory disease and was only evaluable by PET. Importantly, unlike previous CAR-T therapies, P-BCMA-101 is demonstrating exceptional safety, with only one instance of suspected cytokine release syndrome (9%) that was minimal and short-lived. No patients demonstrated neurotoxicity and no patients required admission to an intensive care unit or treatment with tocilizumab or steroids, interventions typically required during episodes of CRS elicited by other CAR-T therapies. Enrollment continues in the higher dose cohorts.

“The latest data results show that P-BCMA-101 induces deep responses in a heavily pretreated population with relapsed/refractory multiple myeloma, with some patients reaching VGPR and even stringent CR at early efficacy assessments,” said Eric Ostertag, M.D., Ph.D., chief executive officer of Poseida Therapeutics. “We believe our advantages of a purified product, where all cells express the CAR molecule, and a product with high levels of stem cell memory T cells, producing a more gradual and prolonged immune response against tumor cells, provide a significantly better therapeutic index when compared with other CAR-T therapeutics. We are also encouraged that P-BCMA-101 is demonstrating significant efficacy even at doses that have been ineffective for other anti-BCMA CAR-T therapies and that our response rates continue to improve as the dose increases.”

P-BCMA-101 Clinical Data Presented as Late Breaker Today at CAR-TCR Summit

As of August 10th, 2018, eleven patients had been treated across three doses groups with average CAR-T cell doses of 51×10^6 (n=3), 152×10^6 (n=7), and 430×10^6 (n=1). These patients were heavily pretreated with a median of 6 prior therapies. The median age was 60, with 73% considered high-risk, including those with high-risk cytogenetics. Peak T-cell expansion was observed between days 14 and 21, which is more gradual than the 5-14 day peak expansion seen with other CAR-T therapies and was associated with less acute cytokine release and other adverse effects.

A favorable safety profile continues to be observed. The single instance of suspected cytokine release syndrome occurred in a patient in the second cohort and rapidly resolved without treatment with tocilizumab or steroids. Consistent with the minimal evidence of CRS symptoms, patients’ biomarkers of CRS, including IL-6 levels, are demonstrating peak levels lower than those seen in patients from other anti-BCMA CAR-T trials. Common adverse events were neutropenia and thrombocytopenia, which are typical for CAR-T and myeloma studies considering the disease, preconditioning chemotherapy and prior chemotherapy regimens. No dose limiting toxicities were observed at any dose. Enrollment requirements

have been achieved in the third dose cohort and enrollment is expected to continue into the fourth and fifth dose cohorts per protocol.

This open-label, multicenter, single-ascending dose, Phase 1 study is designed to assess the safety of P-BCMA-101 in up to 40 subjects with relapsed and/or refractory multiple myeloma. The primary objective of this study is to determine the safety and maximum-tolerated dose of P-BCMA-101. Secondary objectives include anti-myeloma effect of P-BCMA-101. This study is funded in part by the California Institute for Regenerative Medicine. Additional information about the Phase 1 clinical study of P-BCMA-101 is available at www.clinicaltrials.gov using identifier: [NCT03288493](https://clinicaltrials.gov/ct2/show/study/NCT03288493)

Preclinical Presentations at CAR-TCR Summit

A Stem Cell Memory CAR-T Therapy for Epithelial-Derived (MUC1-C) Solid Tumors

- Poseida is developing an autologous stem cell memory CAR-T therapy targeting the C terminal region of Mucin-1 (MUC1-C), which is highly expressed on most epithelial-derived cancers, including breast, lung, renal cell, colorectal, ovarian and pancreatic cancers, among others.
- P-MUC1C-101 includes the same features of Poseida's autologous P-BCMA-101 CAR-T therapy, including a selection gene to create a pure CAR-positive product, a high level of stem cell memory T-cells, and a safety switch.
- P-MUC1C-101 elicited tumor elimination across multiple tumor types, including rapid and complete elimination of established tumors in a mouse xenograft model of breast cancer.
- Oral presentation by Dr. Devon J. Shedlock, vice president of preclinical development at Poseida
- CAR-TCR Discovery Track; September 6th, 2:35 p.m. ET

Manufacture of Allogeneic "Universal Donor" CAR-T Therapies using piggyBac™ and Cas-CLOVER™ Gene Editing Technologies

- Poseida is developing an allogeneic, or universal donor, CAR-T Therapy called P-BCMA-ALLO1 targeting BCMA. It includes the same features of Poseida's autologous P-BCMA-101 CAR-T therapy, including a selection gene for product purity, a high level of stem cell memory T-cells, and a safety switch.
- TCR and MHC1 are knocked out using Poseida's Cas-CLOVER™ high-fidelity gene editing system, eliminating alloreactivity (both graft-versus-host and host-versus-graft) to below the limit of detection.
- Integration of Poseida's "booster molecules" demonstrated ability to mitigate potential deficiencies of TCR knock out in CAR-T cells and successfully improves T-cell expansion.
- P-BCMA-ALLO1 cells exhibit comparable levels of BCMA-specific CAR expression and comparable antigen-specific functionality to the autologous P-BCMA-101 product currently under clinical investigation.
- Oral presentation by Dr. Burton E. Barnett, research scientist at Poseida
- CAR-TCR Manufacturing Track; September 6th, 4:10 p.m. ET

How Can We Create An Allogeneic, Virus Free Manufacturing Process?

- Panel discussion by Dr. Burton E. Barnett, research scientist at Poseida
- CAR-TCR Manufacturing Track; September 6th, 2:35pm ET

PSMA-specific CAR-T Memory Stem Cell Therapy Eliminates Solid Tumors in Multiple Prostate Cancer Models

- Poseida is developing an autologous stem cell memory CAR-T therapy targeting PSMA, which is overexpressed in most metastatic prostate cancers.
- P-PSMA-101 includes the same features of Poseida's autologous P-BCMA-101 CAR-T therapy, including a selection gene for product purity, a high level of stem cell memory T-cells, and a safety switch.
- The Centyrin binder used to construct the P-PSMA-101 CAR exhibited exquisite specificity for PSMA following a study interrogating an extensive library of over 5,400 proteins expressed on the surface of human cells.
- P-PSMA-101 elicited tumor elimination in two different mouse xenograft solid tumor models including one implanted with human LNCaP, a human cell line derived from castrate-resistant metastatic prostate cancer that endogenously expresses PSMA and another modeling bone metastasis using a second cell line derived from castrate-resistant metastatic prostate cancer, called PC-3, which was engineered to express PSMA.
- Poster presentation by Dr. Jenessa B. Smith, research scientist at Poseida
- Scientific Poster Session; September 6th, 8:00 a.m. ET

About P-BCMA-101

P-BCMA-101 is a CAR-T immunotherapy designed to supercharge a patient's own T cells to safely and effectively eliminate tumor cells carrying B cell maturation antigen (BCMA), which is expressed on essentially all multiple myeloma tumor cells. P-BCMA-101 modifies a patient's T cells using piggyBac™, which enables several desirable features, including:

- **T stem cell memory:** P-BCMA-101 is comprised of a high level of stem cell memory T-cells (Tscm), resulting in unprecedented durability of response without re-administration of product in multiple preclinical studies.
- **Pure product:** The addition of a human-derived positive selection gene results in a product that is composed almost entirely of modified CAR-T cells in contrast with lentivirus-based products, which are generally 5-30% pure. The higher purity of the product may also result in less toxicity.
- **Safety:** piggyBac™ is non-oncogenic and has a safer integration profile than lentivirus. In addition, a human-derived safety switch is added such that P-BCMA-101 can be rapidly attenuated or eliminated if significant side effects occur.

About Poseida Therapeutics, Inc.

Poseida Therapeutics is a clinical-stage biotechnology company translating best-in-class gene engineering technologies into lifesaving cell therapies. The company is developing CAR T-cell immunotherapies for multiple myeloma, prostate and other cancer types, as well as gene therapies for orphan diseases. P-BCMA-101 is Poseida's lead CAR-T therapy currently in Phase 1 clinical development for the treatment of multiple myeloma. Poseida has assembled a suite of industry-leading gene engineering technologies, including the piggyBac™ DNA Modification System, TAL-CLOVER™ and Cas-CLOVER™ site-specific nucleases, and Footprint-Free™ Gene Editing (FFGE). For more information, visit www.poseida.com.

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