Poseida Announces Initial Phase 1 Data for P-BCMA-101 CAR-T Stem Cell Memory Product in Patients with Relapsed/Refractory Multiple Myeloma

Low dose cohort completed with rapid and clear signs of efficacy in all three patients, and no cytokine release syndrome

Data presented at the American Association for Cancer Research (AACR) Annual Meeting 2018

SAN DIEGO, April 17, 2018 (GLOBE NEWSWIRE) -- Poseida Therapeutics Inc. ("Poseida"), a San Diego-based company translating best-in-class gene engineering technologies into lifesaving cell therapies, announced initial data from the lowest dose cohort of its ongoing Phase 1 study of its P-BCMA-101 CAR-T product in relapsed/refractory multiple myeloma. All three patients remain on study with efficacy seen in all three, including at least one partial response lasting more than 10 weeks at time of data cutoff, and no dose limiting toxicities and no cytokine release syndrome (CRS) of any grade. Additional patients are now being enrolled in the next dose cohort.

The poster presentation (abstract: CT130), titled **"**Clinical trial of P-BCMA-101 T stem cell memory (Tscm) CAR-T cells in relapsed/refractory (r/r) multiple myeloma (MM)," is being presented at the American Association for Cancer Research (AACR) Annual Meeting on Tuesday, April 17, 2018 from 8:00 a.m. to 12:00 p.m. CT.

"The results from the first cohort of the Phase 1 P-BCMA-101 study have surpassed historical benchmarks of safety and efficacy in multiple myeloma at this dose level and give us confidence to move ahead into additional dose cohorts," said Eric Ostertag, M.D., Ph.D., chief executive officer of Poseida Therapeutics.

In prior clinical trials by other groups testing other BCMA-targeted CAR-T therapies at similar low doses, there were few responses and no response lasted longer than eight weeks. Also, the majority of patients, both responders and non-responders, experienced CRS symptoms. By contrast, in Poseida's study, the first patient showed a partial response just two weeks following treatment and the response has continued more than 10 weeks, marking the longest duration of response reported with an anti-BCMA CAR-T at this dose level. Two additional patients have demonstrated similar, clear and measurable signs of tumor regression.

"The lack of cytokine release syndrome (CRS) in any of the three patients in spite of marked efficacy is unprecedented at this dose, which we believe is attributable to multiple differentiated aspects of our technology resulting in a highly purified CAR-T product with a high percentage of cells with a T stem cell memory phenotype," Dr. Ostertag said.

As of April 3, 2018, three patients had been treated in the low dose cohort of 0.75 x 10^6 P-BCMA-101+ CAR-T cells/kg. These patients were heavily pretreated with 6-9 prior therapies. The first patient, who has a lambda light chain myeloma, achieved partial response (PR) at 2 weeks post-treatment, which continues through at least week 12 and demonstrating a maximal reduction in urine M-protein of 92% and in plasma free light chains (FLCs) of 79%. In the second patient, who has an oligosecretory myeloma with M-protein and FLCs within normal limits, all bone lesions present at time of P-BCMA-101 administration resolved to below background between weeks 4 and 8 by PET imaging. In the third patient, who has a lambda light chain myeloma, urine M-protein and FLCs briefly dipped and rose following bridging chemotherapy, then markedly decreased at 4-weeks post-P-BCMA-101 administration, corresponding with P-BCMA-101 expansion in the peripheral blood.

A favorable safety profile was observed with no clear CRS symptoms or significant increase in CRS biomarkers seen in any patient despite marked anti-myeloma activity in all patients. 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, and the dose was escalated to 2 x 10^6 P-BCMA-101+ CAR-T cells/kg for the next patient cohort. The first patient has subsequently been treated at this dose level, with no CRS yet reported.

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

The poster will be available on the publications page of Poseida's website at www.poseida.com/publications.

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 an exceptionally high proportion 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 essentially 100% pure in contrast with lentivirus-based products, which are generally 5-30% pure. The higher purity of the product may 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 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.

Corporate Communications Contact:
Jason Spark
Canale Communications
619-849-6005
Jason@canalecomm.com