Multiple Programs Featuring Poseida Therapeutics' Advanced CAR-T Therapies Presented at American Society of Hematology 2017 Annual Meeting

December 11, 2017

SAN DIEGO, Dec. 11, 2017 (GLOBE NEWSWIRE) -- Poseida Therapeutics Inc. ("Poseida"), a San Diego-based company translating best-in-class gene engineering technologies into lifesaving cell therapies, today announced preclinical data from multiple chimeric antigen receptor T cell (CAR-T) programs in Poseida's drug development pipeline.

"Our findings continue to demonstrate that our advanced gene engineering technologies, including our piggyBac™ DNA Modification System and our Cas-CLOVER™ gene editing, are keys to the production of pure modified CAR-T cells with low-to-no off-target effects, a predominantly high T stem cell memory (Tscm) phenotype, and prolonged durability," said Eric Ostertag, M.D., Ph.D., chief executive officer of Poseida. "Our modified CAR-T cells demonstrate potential to overcome many limitations of first generation CAR-T therapies with poor durability, and open doors to entirely new approaches to treatments such as a universal T-cell therapy for cancer, as well as a CAR-T pre-conditioning approach for stem cell transplantation procedures."

P-BCMA-101: autologous anti-BCMA CAR-T stem cell memory therapy
Poster Presentation: B-Cell Maturation Antigen (BCMA)-Specific, Centyrin™-Based, PiggyBac™-Transposed CAR-T Memory Stem Cells Are Effective Against p53-/- and Patient-Derived Multiple Myeloma Tumors *(presented by M.D. Anderson Cancer Center)*
Abstract Number: 3068
Date and Time: 6:00 p.m. to 8:00 p.m. ET, Sunday, December 10, 2017

- P-BCMA-101 CAR molecules were designed with a safety switch, a fully human BCMA-specific binder, and a selection gene for manufacturing nearly 100% pure modified CAR-T cells
- P-BCMA-101 was comprised predominately of long-lived T stem cell memory cells (60-80% Tscm), a T-cell subtype correlated with durability of response
- Potent anti-tumor activity was seen in vitro and in vivo against highly aggressive multiple myeloma cell lines and tumors obtained from multiple myeloma patients, including rapid elimination of tumors in a patient-derived xenograft model within 3-7 days, with all mice surviving at study completion

P-BCMA-ALLO1: allogeneic (universal) anti-BCMA CAR-T stem cell memory therapy
Oral Presentation: Production of Universal Anti-BCMA CAR-T Cells with Reduced Alloreactivity, but Potent Effector Function for the Treatment of Multiple Myeloma
Abstract Number: 503
Date and Time: 5:30 p.m. ET, Sunday, December 10, 2017

- Cas-CLOVER™ (dCas9 fused to Clo51 nuclease) hybrid gene editing system capable of developing a high fidelity, off-the-shelf CAR-T cell product
- P-BCMA-ALLO1 did not show alloreactivity, potentially eliminating the problem of graft vs. host disease and graft rejection, thereby making it a true "universal donor" CAR-T product
- More than 95% of P-BCMA-ALLO1 was comprised of modified T-cells, and the majority of those cells were the highly desirable early memory T cells subset (Tscm) that exhibits the greatest capacity for self-renewal
- Data demonstrate that P-BCMA-ALLO1 exhibited significantly reduced alloreactivity yet retained potent effector function

Hematopoietic Stem Cell (HSC) CAR-T Therapy: novel conditioning strategy for stem cell transplant
Poster Presentation: The Application of 'Drug-Reversible' CAR-T Cells Directed Against Recipient Hematopoietic Cells as a Selective Conditioning Strategy for Stem Cell Transplantation
Abstract Number: 1893
Date and Time: 5:30 p.m. to 7:30 p.m. ET, Saturday, December 9, 2017

- Large carrying capacity of piggyBac™ DNA Modification System allows for design of a CAR molecule targeting CD117 (c-kit) and/or CD133, with the integration of a safety switch, as well as a selection marker for manufacturing of pure product
- Manufacturing process yielded CAR-T cells that were mainly of Tscm phenotype
- CAR-T cells were capable of specific depletion of c-kit- or CD133-expressing target cells
- Lead CAR candidates were identified for a novel conditioning strategy to eliminate recipient hematopoietic stem cells in the bone marrow and then deplete CAR-T cells prior to engraftment of donor or gene-corrected stem cells for stem cell transplant procedures

About Cas-CLOVER™ Gene Editing Technology
Cas-CLOVER™ (previously called NextGEN™ CRISPR) is a proprietary hybrid gene editing technology for the design of next generation cell and gene therapies. It combines the ease of use and low cost of CRISPR with high fidelity editing of other nucleases. CLOVER™ uses a differentiated site-specific nuclease (Clo51) as its DNA cutting mechanism, which only works as two halves that must simultaneously dimerize on both side of a target site in order to cut. Cas-CLOVER™ is targeted using a set of two distinct guide RNAs similar to CRISPR/Cas9, but with the exquisite specificity of type IIS nucleases, resulting in no to very low off target mutations.

About Poseida Therapeutics Inc.
Poseida Therapeutics is a clinical-stage 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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