

Poseida Therapeutics Presents Preclinical Data Demonstrating Tumor Response from BCMA-Specific CAR-T Program at American Society of Hematology Annual Meeting

SAN DIEGO, December 5, 2016 – Poseida Therapeutics Inc. (“Poseida”), a San Diego-based company translating best-in-class gene editing technologies into lifesaving therapeutics, today announced preclinical data from the company’s B-cell maturation antigen (BCMA) specific chimeric antigen receptor (CAR) T-cell drug candidate, referred to as P-BCMA-101. Data demonstrated a superior stem cell memory phenotype and self-renewal capacity, as well as strong and persistent anti-tumor activity in an established xenograft model of multiple myeloma. The company plans to initiate a Phase 1 clinical trial of P-BCMA-101 for the treatment of patients with relapsed or refractory multiple myeloma in 2017. These data were featured across three presentations at the American Society of Hematology (ASH) Annual Meeting taking place December 3-6, 2016.

“CAR-T therapies have demonstrated potent activity against blood cancers, but their potential has been limited by a lack of durable and persistent responses,” said Eric Ostertag, M.D., Ph.D., chief executive officer of Poseida. “In the studies presented at ASH, we report multiple desirable attributes in a single CAR-T product for the first time, including a more stable and potentially less immunogenic binding domain, a high proportion of cells with stem cell memory, a safety switch and a manufacturing process that consistently yields pure CAR-expressing T cells.”

The studies explore the utility and performance of Poseida’s proprietary CAR-T product made using the company’s piggyBac™ non-viral gene delivery system and Centyrin™ binding scaffold. Key characteristics of P-BCMA-101 featured in the presentations include:

- Centyrin™ antigen binding scaffolds are viable replacements to the single chain (scFv) domains used with first-generation CARs. Centyrins are smaller, more stable binding molecules, with no evidence of tonic signaling or T-cell exhaustion that limit durability of existing immunoglobulin-based CAR binding domains.¹ Centyrins are also fully human proteins that are potentially less likely to illicit an immune response compared to rodent-derived binders.
- P-BCMA-101 exhibited significant stem cell memory, a highly desirable characteristic for CAR-T therapies, creating a stable population of viable memory T cells to prevent cancer relapse and resulting in potent and durable responses. On average, 70-80% of P-BCMA-101 T cells exhibited a stem cell memory phenotype in direct contrast to lentivirus-derived products, which resulted in a low proportion of stem cell memory.² In a mouse xenograft model implanted with an aggressive human multiple myeloma cell line, P-BCMA-101 eliminated the tumor in 100% of treated animals. Importantly, tumor relapses were also eliminated, resulting in a highly durable response.
- The manufacturing process of P-BCMA-101 from primary human T cells is facile and scalable, employs no virus, cytokines or magnetic beads, and it consistently produced more than enough modified T cells to treat patients. P-BCMA-101 is manufactured using a proprietary non-viral transposon (piggyBac™) capable of delivering 30 times more cargo than traditional virus-based CAR T-cell modification systems. The enormous cargo capacity enables incorporation of a Centyrin™ binding domain, a gene for selection of CAR-expressing T cells, and a safety switch for rapid depletion of T cells in the cases of adverse events.³

Poster presentations are available on the publications page of Poseida’s website at www.poseida.com/publications.

About Poseida Therapeutics, Inc.



Poseida Therapeutics is translating best-in-class gene editing technologies into lifesaving treatments. The company is developing CAR T-cell immunotherapies for multiple myeloma and other cancer types, as well as gene therapies for orphan diseases. Poseida has assembled a suite of industry-leading gene editing technologies, including the piggyBac™ DNA Modification System, XTN™ TALEN and NextGEN™ CRISPR site-specific nucleases, and Footprint-Free™ Gene Editing (FFGE). For more information, visit www.poseida.com.

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References

1. Barnett B.E., *et al.* Development of Novel Non-Immunoglobulin Centyrin™-Based CARs (CARTyrins) Targeting Human BCMA; (Abstract 4557). Presented at the 58th Annual Meeting of The American Society of Hematology, December 5, 2016 in San Diego, CA. hematology.org.
2. Barnett B.E., *et al.* piggyBac™-Produced CAR-T Cells Exhibit Stem-Cell Memory Phenotype; (Abstract 2167). Presented at the 58th Annual Meeting of The American Society of Hematology, December 3, 2016 in San Diego, CA. hematology.org.
3. Hermanson D.L., *et al.* A Novel BCMA-Specific, Centyrin™-Based CAR-T Product for the Treatment of Multiple Myeloma; (Abstract 2127). Presented at the 58th Annual Meeting of The American Society of Hematology, December 3, 2016 in San Diego, CA. hematology.org.