Poseida Therapeutics Presents Encouraging Preclinical Data Across its CAR-T and Gene Therapy Programs at the American Society of Gene and Cell Therapy 2021 Virtual Annual Meeting

Preclinical data demonstrates potential of piggyBac® DNA delivery system in developing gene therapies for the treatment of genetic liver disorders

P-BCMA-ALL01, Poseida's first allogeneic CAR-T candidate targeting BCMA for relapsed/refractory (R/R) multiple myeloma, demonstrates potent activity in preclinical models

Preclinical data support anti-c-kit CAR-T as a preconditioning therapy for the transplantation of hematopoietic stem cells in patients with acute myeloid leukemia (AML)

SAN DIEGO, May 11, 2021 /PRNewswire/ -- Poseida Therapeutics, Inc. (Nasdaq: PSTX), a clinical-stage biopharmaceutical company utilizing proprietary genetic engineering platform technologies to create cell and gene therapeutics with the capacity to cure, today will give multiple oral and poster presentations at the American Society of Gene and Cell Therapy 2021 Virtual Annual Meeting being held May 11-14, 2021.

The Company's oral presentation will highlight new data demonstrating the potential of its proprietary piggyBac DNA Delivery System for the treatment of genetic liver disorders in children and infants. Two additional presentations will highlight preclinical data supporting Poseida's first allogeneic CAR-T product candidate, P-BCMA-ALLO1 for R/R multiple myeloma, as well as preclinical data supporting the Company's anti-c-kit CAR-T program as a potentially safer preconditioning regimen for hematopoietic stem cell transplantation in patients with AML.

"At Poseida, we are applying our proprietary technology platforms to develop the next wave of cell and gene therapies to not only treat severe cancers but to also unlock the potential of single treatment cures," said Eric Ostertag, M.D., Ph.D., Chief Executive Officer. "The preclinical data being presented today further validate our CAR-T approach in multiple myeloma and in preconditioning for patients with AML, as well as demonstrate the exciting potential of our liver directed gene therapies, particularly in juvenile patients."

Presentation Highlights:

Oral Presentation: "Preclinical Evaluation of Combined Adeno-Associated Virus and Nanoparticle Delivery of piggyBac Transposon System for Durable Transgene Expression in the Growing Neonatal Murine Liver"
Session Date/Time: Tuesday, May 11, 2021, 5:45pm - 6:00pm ET
Abstract Number: 30

In a preclinical study, Poseida evaluated concomitant delivery of recombinant adeno-associated virus (rAAV) vectors and novel nanoparticle (NP) vectors using its piggyBac and "Super" piggyBac (SPB) technologies in order to deliver transposon and transposase in a growing neonatal mouse model. Data demonstrated that the piggyBac DNA Delivery System was effective in using both rAAV and NP vectors to introduce edited genes into targeted hepatocyte genomes. Poseida also found that SPB, a hyperactive form of the transposase, produced stable vector integration into the hepatocyte genome for more than three months, compared to transpose alone. Similarly, delivery of a novel NP formulation using SPB produced efficient delivery of mRNA to the liver hepatocytes, with similarly high levels of durability in the transgene expression. Taken together, these preclinical findings suggest the potential of piggyBac and SPB technology for gene therapies that treat congenital liver disease in infants and young children.

Poster Presentation: "P-BCMA-ALL01: A Fully Allogeneic Stem Cell Memory T Cell (TSCM) CAR-T Therapy Targeting BCMA for the Treatment of Multiple Myeloma Shows Potent Anti-Tumor Activity"
Session Date/Time: Tuesday, May 11, 2021, 8:00am – 10:00am ET
Abstract Number: 789

P-BCMA-ALLO1 is Poseida's first fully allogenic product candidate targeting B-cell maturation antigen (BCMA) for the treatment of relapsed/refractory multiple myeloma. In in vitro and in vivo preclinical studies, P-BCMA-ALL01 showed effective, targeted cancer cell killing and cytokine secretion, with similar or superior performance in anti-tumor efficacy compared to an autologous CAR-T therapy. Inclusion of a proprietary "booster molecule" in the allogeneic manufacturing process further improved expansion of gene-edited cells and enabled production of hundreds of patient doses from a single manufacturing run, thereby reducing the manufacturing cost per dose into the same range as that of a monoclonal antibody.

Poster Presentation: "Anti-c-kit CAR-T Cells Afford Effective Eradication of Human AML and Normal Hematopoietic Cells in a Preclinical Model of Safer Non-Genotoxic Stem Cell Transplant Conditioning"
Session Date/Time: Tuesday, May 11, 2021, 8:00am – 10:00am ET
Abstract Number: 715

Poseida is investigating its anti-c-kit CAR-T program, which leverages its proprietary piggyBac DNA Delivery System in preclinical studies as a potentially safer precursor conditioning therapy to the transplantation of hematopoietic stem cells (HSC) for patients suffering from AML. The piggyBac delivery vectors under investigation include a transposon that generates pure CAR+ product as well as a safety switch that allows rapid clearance of the reactive CAR-T cells prior to donor transplant of hematopoietic stem cells. Preclinical data to be presented in the poster showed that the lead CAR-T cells that express the anti-c-kit binder (CAR 1) deplete up to 92% of human CD34+ stem and progenitor cells in bone marrow within 48 hours. Additionally, an enhanced anti-c-kit CAR-T product, CAR 2, killed an estimated >99% of leukemia cells, exceeding the killing ability of a single dose of 30 mg/kg dose of busulfan. These encouraging data suggest that stem cell-directed CAR-T cells may be a safer preconditioning regimen compared to the current standard of care and may expand access to treatment for acute myeloid leukemia patients needing HSC transplant.

About Poseida Therapeutics, Inc.
Poseida Therapeutics is a clinical-stage biopharmaceutical company dedicated to utilizing our proprietary genetic engineering platform technologies to create next generation cell and gene therapeutics with the capacity to cure. We have discovered and are developing a broad portfolio of product candidates in a variety of indications based on our core proprietary platforms, including our non-viral piggyBac® DNA Delivery System, Cas-CLOVER™ Site-specific Gene Editing System and nanoparticle- and AAV-based gene delivery technologies. Our core platform technologies have utility, either alone or in combination, across many cell and gene therapeutic modalities and enable us to engineer our wholly-owned portfolio of product candidates that are designed to overcome the primary limitations of current generation cell and gene therapeutics. To learn more, visit www.poseida.com and connect with us on Twitter and LinkedIn.

Forward-Looking Statements
Statements contained in this press release regarding matters that are not historical facts are "forward-looking statements" within the meaning of the Private Securities Litigation Reform Act of 1995. Such forward-looking statements include statements regarding the clinical data presented, the potential benefits of Poseida's technology platforms and product candidates and Poseida's plans and strategy with respect to developing its technologies and product candidates. Because such statements are subject to risks and uncertainties, actual results may differ materially from those expressed or implied by such forward-looking statements. These forward-looking statements are based upon Poseida's current expectations and involve assumptions that may never materialize or may prove to be incorrect. Actual results could differ materially from those anticipated in such forward-looking statements as a result of various risks and uncertainties, which include, without limitation, risks and uncertainties associated with development and regulatory approval of novel product candidates in the biopharmaceutical industry, the fact that future clinical results could be inconsistent with results observed to date and the other risks described in Poseida's filings with the Securities and Exchange Commission. All forward-looking statements contained in this press release speak only as of the date on which they were made. Poseida undertakes no obligation to update such statements to reflect events that occur or circumstances that exist after the date on which they were made, except as required by law.

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Marcy Graham, VP, Corporate Affairs, Poseida Therapeutics, Inc., 858-779-3108, mgraham@poseida.com; Sarah Thailing, Director, Corporate Communications and IR, 858-605-3717, sthailing@poseida.com