Poseida Therapeutics Presents Encouraging Preclinical Data Across its Gene Therapy Programs at the American Society for Gene and Cell Therapy 2023 Annual Meeting

Six presentations highlight significant advancements across the Company's Gene Therapy platform

Preclinical data supports potential of P-OTC-101 to correct severe Ornithine Transcarbamylase Deficiency (OTCD) following a single dose

Data presented establishes proof-of-concept for site-specific knock-in of a transgene using Cas-CLOVER™, Poseida's high-precision gene editing technology

SAN DIEGO, May 18, 2023 /PRNewswire/ -- Poseida Therapeutics, Inc. (Nasdaq: PSTX), a clinical-stage cell and gene therapy company advancing a new class of treatments for patients with cancer and rare diseases, today highlights its two oral and four poster presentations on the Company's preclinical gene therapy programs and platforms at the American Society of Gene and Cell Therapy (ASGCT) 2023 Annual Meeting, being held at the Los Angeles Convention Center in Los Angeles and virtually May 16 – 20, 2023.

"We are excited to highlight multiple advancements across our gene therapy programs and platform technologies at ASGCT, which underscore the significant progress our company has made in the past year," said Brent Warner, President, Gene Therapy at Poseida Therapeutics. "We are making encouraging progress with our P-OTC-101 program, where our hybrid LNP+AAV approach has shown its potential to deliver a functional cure for severe, pediatric-onset OTCD with a single dose. In addition, early preclinical data utilizing our Cas-CLOVER and non-viral piggyBac® technologies continue to show great promise as we seek to unlock the potential of non-viral gene therapies for patients."

Oral Presentations

Title: *Preclinical Proof-of-Concept: A Novel Hybrid Gene Therapy Approach to Treat Severe Early-Onset Ornithine Transcarbamylase Deficiency*
Session Title: Metabolic, Storage, Endocrine, Liver and Gastrointestinal Diseases I
Presentation Time: Thursday, May 18, 2023, 2:30 – 2:45 PM PST
Location: Room 403 AB
Abstract Number: 127

Ornithine Transcarbamylase Deficiency (OTCD) is an X-linked urea cycle disorder that prevents the breakdown and excretion of ammonia, allowing it to rise to toxic levels and affect the central nervous system, leading to coma, seizures, brain damage, and death. Poseida has developed P-OTC-101, a liver directed gene therapy utilizing a hybrid lipid-nanoparticle (LNP) and adeno-associated virus (AAV) delivery system based on its piggyBac® DNA insertion system to enable integration of the therapeutic human OTC gene into the genome. In this study, researchers demonstrated correction of severe OTCD following a single dose in a stringent mouse model of the disease. Researchers also reported preclinical pharmacology showing dose-response behavior as well as favorable translational safety and pharmacology in mice and non-human primates using this hybrid platform approach, which supports further development of P-OTC-101 towards evaluation in humans.

Title: *Cas-CLOVER Technology Enables Precise Gene Editing and Site-Specific Transgene Insertion in Mouse Liver*
Session Title: Gene Targeting and Gene Correction: Liver
Presentation Time: Thursday, May 18, 2023, 3:00 – 3:15 PM PST
Location: Room 515 AB
Abstract Number: 157

This presentation highlights the potential of Cas-CLOVER, Poseida's high-fidelity, proprietary gene editing technology co-formulated with guide RNAs as a single LNP. Data demonstrated highly efficient editing and favorable tolerability in mice following a single dose of Cas-CLOVER with extremely low off-target editing in the liver. The study establishes proof-of-concept for knock-in of a transgene using Cas-CLOVER and a fully non-viral delivery system in mice and further supports the potential of this technology to develop effective therapies for rare diseases.

Poster Presentations

Title: *Demonstration of Human Factor VIII Expression and Activity Following Single and Repeat Dosing of a Non-Viral Integrating Gene Therapy*
Session Title: Wednesday Poster Session
Session Date/Time: Wednesday, May 17, 2023, 12:00 PM PST
Location: West Hall A
Abstract & Poster Board Number: 638

P-FVIII-101 is a fully non-viral liver-directed gene therapy combining Poseida's proprietary piggyBac® technology with nanoparticle delivery for the treatment of Hemophilia A. This study demonstrated the potential of P-FVIII-101 to produce durable human FVIII expression over six months in an adult mouse model of severe Hemophilia A following a single dose. The study also highlighted the potential of repeat dosing to achieve therapeutic levels of human FVIII activity. An integration site analysis revealed a favorable insertion profile and well-controlled integrated vector copy number. These data provide proof-of-principle evidence toward a potential functional cure for Hemophilia A.

Title: *Development of a Novel Non-Viral Gene Therapy Platform*
Session Title: Thursday Poster Session
Session Date/Time: Thursday, May 18, 2023, 12:00 PM PST
Location: West Hall A
Abstract & Poster Board Number: 945

The piggyBac® DNA insertion system is a transposon-based gene therapy platform that enables stable integration of the therapeutic transgene into the genome, thereby offering the potential for durable and lifelong activity. This poster details formulation discovery work on an LNP comprising a novel degradable ionizable lipid with unique capabilities for efficient DNA delivery to the liver, as well as discovery and optimization of an LNP-based delivery system capable of co-encapsulating mRNA and DNA for delivery of piggyBac® transposon system components.

Title: *Editing of a γ-Globin (HBG1/HBG2) cis-Regulatory Element in Human Hematopoietic Stem and Progenitor Cells Using Cas-CLOVER™ Technology Reactivates Fetal Hemoglobin*
Session Title: Thursday Poster Session
Session Date/Time: Thursday, May 18, 2023, 12:00 PM PST
Location: West Hall A
Abstract & Poster Board Number: 1212

This study demonstrates that high-fidelity Cas-CLOVER nuclease targeting of gamma globin genes provides efficient editing and reactivation of fetal hemoglobin expression. Cas-CLOVER-mediated gene editing of gamma globin genes was also shown to produce up to 70% positivity for fetal hemoglobin among differentiated red blood cells. Further, Cas-CLOVER-mediated gene editing did not adversely affect stem cell capabilities, including potential to produce the red blood cell lineage. These data support Cas-CLOVER editing of gamma globin genes as a potential therapeutic strategy for genetic diseases such as β-thalassemia and sickle cell disease.

Title: *Development and Optimization of Novel Super piggyBac®-Based Hybrid Gene Therapy Approach*
Session Title: Friday Poster Session
Session Date/Time: Friday, May 19, 2023, 12:00 PM PST
Location: West Hall A
Abstract & Poster Board Number: 1318

This presentation describes the discovery and optimization of a novel LNP formulation suitable for delivery of Poseida's super piggyBac® transposase to the liver. In addition, these studies highlighted in vivo safety and pharmacology of Poseida's lead LNP formulation in mice and non-human primates for use in liver-directed hybrid gene therapy applications. The presentation further characterizes the use of Poseida's hybrid gene therapy platform in a mouse model of OTCD to achieve disease resolution at significantly lower doses of AAV. These data demonstrate the versatility of the piggyBac® hybrid platform and its potential to achieve durable transgene expression when administered early in life.

About Poseida Therapeutics, Inc.

Poseida Therapeutics is a clinical-stage biopharmaceutical company advancing differentiated cell and gene therapies with the capacity to cure certain cancers and rare diseases. The Company's pipeline includes allogeneic CAR-T cell therapy product candidates for both solid and liquid tumors as well as in vivo gene therapy product candidates that address patient populations with high unmet medical need. The Company's approach to cell and gene therapies is based on its proprietary genetic editing platforms, including its non-viral piggyBac® DNA Delivery System, Cas-CLOVER™ Site-Specific Gene Editing System and nanoparticle and hybrid gene delivery technologies. The Company has formed global strategic collaborations with Roche and Takeda to unlock the promise of cell and gene therapies for patients. Learn more at www.poseida.com and connect with Poseida 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, among other things, expected plans with respect to clinical trials, including timing of regulatory submissions and approvals and clinical data updates; anticipated timelines and milestones with respect to the Company's development programs and manufacturing activities and capabilities; the potential capabilities and benefits of the Company's technology platforms and product candidates; and the Company'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 the Company'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, the Company's reliance on third parties for various aspects of its business; risks and uncertainties associated with development and regulatory approval of novel product candidates in the biopharmaceutical industry; the Company's ability to retain key scientific or management personnel; the fact that the Company will have limited control over the efforts and resources that its strategic partners devote to advancing development programs under their respective collaboration agreements and the Company may not receive the potential fees and payments under the collaboration agreements and the ability of its strategic partners to early terminate the collaborations, such that the Company may not fully realize the benefits of such collaborations; and the other risks described in the Company'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. The Company 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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