



Poseida Therapeutics Hosts Gene Therapy R&D Day Highlighting New Scientific Advancements and Pipeline Focus

Fully non-viral approach to genetic medicine employs differentiated gene delivery, gene editing and gene insertion technology

Progressing two fully non-viral programs in rare disease with significant unmet patient need

Virtual R&D Day featuring academic experts and Poseida's leadership and scientific teams to be held today at 10:00am ET / 7:00am PT

SAN DIEGO, April 17, 2024 /PRNewswire/ -- Poseida Therapeutics, Inc. (Nasdaq: PSTX), a clinical-stage cell therapy and genetic medicines company advancing a new class of treatments for patients with cancer and rare diseases, today announced that the Company plans to highlight progress across its proprietary non-viral genetic engineering and delivery platform and rare disease pipeline during a virtual R&D Day to be held today at 10:00am ET / 7:00am PT.

"Poseida is forging ahead with a renewed focus on our genetic medicine portfolio. Our system of proprietary, non-viral tools used individually or together has the capacity to treat rare genetic diseases as well as address much more prevalent diseases," said Kristin Yarema, Ph.D., President & Chief Executive Officer of Poseida Therapeutics. "In the short-term, we are laser-focused on progressing our two lead non-viral candidates within areas of significant opportunity: P-KLKB1-101 for Hereditary Angioedema, and P-FVIII-101, which utilizes our fully non-viral stable gene insertion technique to treat Hemophilia A, a condition affecting approximately 30,000 adults and children in the U.S. alone."

"Poseida has developed a broad suite of fully non-viral, differentiated genetic engineering technologies, including stable, potentially site-specific insertion of whole genes, high-fidelity gene editing, and strength in delivery systems including lipid nanoparticles," said Blair Madison, Ph.D., Chief Scientific Officer of Gene Therapy at Poseida Therapeutics. "We believe this uniquely positions us in the industry to deliver on the hope and promise of genetic medicines and bring much-needed therapies to patients in need."

The event will highlight the Company's proprietary genetic engineering and delivery platform, including its non-viral gene insertion and gene editing programs. External expert speakers will include:

- Marc Riedl, M.D., M.S., Professor of Medicine and Clinical Director of the U.S. HAEA Angioedema Center at University of California, San Diego; and
- Steven W. Pipe, M.D., Professor of Pediatrics and Pathology, University of Michigan

Key Gene Therapy R&D Day Topics and Highlights

Gene Therapy Programs

The Company will present advancements in fully non-viral liver-directed gene therapies highlighting the potential for functional cures across commercially viable indications.

- **P-KLKB1-101** is the Company's lead liver-directed investigational gene therapy program for the treatment of hereditary angioedema (HAE), a rare, inherited disorder which results in the swelling of the limbs, intestinal tract, and airways which can be both debilitating and life-threatening. The Company will share data highlighting durable disease correction and high fidelity in pre-clinical studies using the Company's enhanced editing technology, Cas-CLOVER™.
- **P-FVIII-101**, the Company's second non-viral gene therapy program, is a liver-directed investigational in vivo gene therapy for the treatment of Hemophilia A, a hereditary disorder caused by a deficiency in Factor VIII (FVIII) production resulting in excessive bleeding occurring either spontaneously or due to trauma. The Company will share data demonstrating durability and restoration of FVIII deficiency to near-normal levels in adult mouse models.

Technology Innovation in Gene Therapy

The Company will highlight significant advancements in its emerging platform technologies.

- **Site-specific Super piggyBac®** is a single enzyme fusion system for site-specific integration, executing clean DNA gene insertion without double strand breaks, unintended mutations, or the need for DNA repair. The Company will announce that the current molecular evolution of its platform yields a 30-fold improvement of DNA expression and efficient targeted cargo integration at single- and multi-copy sites. These data support the potential for the Company's non-viral insertion technology as an efficient and safe approach to achieve sustained DNA integration and expression to remediate disease.
- **Cas-CLOVER** is a proprietary high-fidelity nuclease for enabling clean site-specific gene editing that is engineered for high specificity. The Company will present data confirming and validating the benefits and advantages of Cas-CLOVER in multiple applications and disease areas.
- **Novel Lipids and DNA Delivery:** The Company is leveraging proprietary lipids notable for their low immunogenicity, dose titration potential, and ability to be manufactured at scale and favorable cost. The Company has achieved multiple breakthroughs in its delivery technology, including novel lipids for in vivo delivery of its technology as well as innovations enabling improved in vivo non-viral DNA delivery.

Video Webcast and Replay

This virtual event and access to the live webcast is available through the following registration link: <https://wsw.com/webcast/cc/pstx6/1466622>.


Registration for this virtual event and access to a replay of the live webcast will be available on the Investors & Media section of www.poseida.com. A replay of the webcast will be available for approximately 90 days following the presentation.

About Poseida Therapeutics, Inc.

Poseida Therapeutics is a clinical-stage biopharmaceutical company advancing differentiated cell therapies and genetic medicines with the capacity to cure certain cancers and rare diseases. The Company's pipeline includes investigational allogeneic CAR-T cell therapies for both solid tumors and hematologic cancers as well as investigational in vivo genetic medicines that address patient populations with high unmet medical need. The Company's approach is based on its proprietary genetic editing platforms, including its non-viral piggyBac[®] DNA Delivery System, Cas-CLOVER[™] Site-Specific Gene Editing System, Booster Molecule and nanoparticle gene delivery technologies, as well as in-house GMP cell therapy manufacturing. The Company has formed a global strategic collaboration with Roche to unlock the promise of cell therapies for patients with hematologic malignancies. Learn more at www.poseida.com and connect with Poseida on [X](#) 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 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, including the efficacy and safety profile of such product candidates; the quotes from Drs. Yarema and Madison; 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; 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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