



## Poseida Therapeutics Presents Preclinical Data from P-FVIII-101 Gene Therapy for Hemophilia A at the 64th ASH Annual Meeting & Exposition

*Data demonstrate that a non-viral, liver-directed gene therapy utilizing Super piggyBac® (SPB) DNA Modification System achieved and maintained normalized human FVIII (hFVIII) activity following a single dose*

*Data establishes preclinical proof of principle for treatment of Hemophilia A across all ages, which could potentially lead to a functional cure*

SAN DIEGO, Dec. 11, 2022 /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 announced that the Company will present preclinical data from its P-FVIII-101 gene therapy program, partnered with Takeda, at the 2022 American Society of Hematology (ASH) Annual Meeting being held in New Orleans and virtually December 10–13, 2022. The data establish preclinical proof of principle for the treatment of Hemophilia A using P-FVIII-101, a non-viral liver-directed gene therapy utilizing Poseida's Super piggyBac delivery system, which could potentially lead to a functional cure.

"We are very excited by these new P-FVIII-101 data, which demonstrate normalization of FVIII levels in an animal model of Hemophilia A," said Brent Warner, President, Gene Therapy at Poseida Therapeutics. "Most importantly, we have demonstrated the use of a fully non-viral gene therapy to address the underlying cause of Hemophilia A, providing key preclinical proof of principle for our program. We look forward to our continued work on this program together with our partner, Takeda."

Details of the oral presentation are as follows:

**Title:** Sustained Factor VIII Activity Following Single Dose of Non-Viral Integrating Gene Therapy

**Presenter:** Brian Truong, Ph.D.

**Presentation Date and Time:** Today, December 11, 2022 at 10:15 AM CT

**Session Name:** 321. Coagulation and Fibrinolysis: Basic and Translational

**Publication Number:** 400

**Location:** Ernest N. Morial Convention Center, 293-294

P-FVIII-101 utilizes the Company's non-viral, nanoparticle-based delivery system together with SPB, which enables increased transgene cargo capacity, stable integration into the genome, potential for re-dosing, and potentially simpler manufacturing processes. The data to be presented show that P-FVIII-101 achieved and sustained normalized (>50%) hFVIII activity following a single dose and delivered therapeutic FVIII activity in mice following single and repeat doses, indicating the potential for dose titration. Durable responses were observed following a single dose reported over the study period of seven months. The data support that with SPB the therapeutic transgene expression cassette can be stably integrated into the genome of liver cells and provide consistent and durable therapeutic activity.

"Although gene therapy has the potential to deliver functional cures for Hemophilia A, current approaches face challenges – both with durability and the ability to re-dose – and are not appropriate for use in juvenile patients," said Denise Sabatino, Ph.D., Research Associate Professor of Pediatrics at the Perelman School of Medicine at the University of Pennsylvania and Children's Hospital of Philadelphia (CHOP) and an author on the oral presentation. "The data being presented today show that P-FVIII-101 has the potential to correct a deficiency in FVIII to near normal levels in juvenile mice, providing a path forward for a more tolerable, durable treatment for Hemophilia A in pediatric patients. Current treatment options are not curative and require lifelong treatment, and P-FVIII-101 may have the potential to significantly improve outcomes for people with Hemophilia A."

In October 2021, Poseida announced that it had entered into a research collaboration and exclusive license agreement with Takeda to utilize the Company's proprietary genetic engineering platform technologies for the research and development of gene therapies, including P-FVIII-101. The companies plan to continue preclinical studies to advance the program toward an Investigational New Drug (IND) application.

### **About P-FVIII-101**

P-FVIII-101 is a liver-directed gene therapy partnered with Takeda combining Poseida's Super piggyBac platform and nanoparticle delivery technologies for the in vivo treatment of Hemophilia A. Hemophilia A is a bleeding disorder caused by a deficiency in Factor VIII production with a high unmet need. P-FVIII-101 utilizes the piggyBac gene integration system delivered via lipid nanoparticle, which has demonstrated stable and sustained Factor VIII expression in animal models.

### **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. Poseida's approach to cell and gene therapies is based on its proprietary genetic editing platforms, including its non-viral Super 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](http://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; 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, Poseida'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; Poseida's ability to retain key scientific or management personnel; 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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