

# Beam Therapeutics to Present New Preclinical Data Highlighting Non-Genotoxic Conditioning Regimens for Patients with Sickle Cell Disease Ahead of Autologous Transplant at the 64th ASH Annual Meeting

November 3, 2022

CAMBRIDGE, Mass., Nov. 03, 2022 (GLOBE NEWSWIRE) -- Beam Therapeutics Inc. (Nasdaq: BEAM), a biotechnology company developing precision genetic medicines through base editing, today announced that new data supporting the advancement of its Engineered Stem Cell Antibody Paired Evasion (ESCAPE) conditioning approach will be presented during poster sessions at the 64<sup>th</sup> Annual American Society of Hematology (ASH) Annual Meeting and Exposition. The meeting is being held December 10-13, 2022, in New Orleans.

Beam is advancing ESCAPE as part of its long-term strategy to support broad accessibility of base editing treatments for patients with sickle cell disease (SCD) and other hematologic diseases. A key component of the company's Wave 2 strategy is focused on improving the safety and tolerability of conditioning regimens, a required pretreatment for patients receiving *ex vivo* gene editing treatment via autologous transplant. To address toxicity challenges associated with currently available conditioning regimens, Beam has leveraged its base editing capabilities to develop a potentially non-genotoxic approach that combines antibody-based conditioning with multiplex gene edited hematopoietic stem cells (HSCs).

The data to be presented at ASH expand upon data presented earlier this year for its ESCAPE-1 approach, which consists of multiplex base edited HSCs that include a therapeutic edit for SCD at the HGB1/2 gene designed to enable upregulation of fetal hemoglobin and an additional simultaneous edit at CD117. Findings showed that the edit-antibody pair targeting CD117, an optimal conditioning target for eliminating HSCs, led edited HSCs to function normally but escape the binding of the conditioning antibody. In addition, Beam will present work on ESCAPE-2, in which the company leveraged its initial ESCAPE work to screen guides that were compatible, in a multiplex-edited HSC, with a next-generation adenine base editor that could install the therapeutic HbG-Makassar edit.

"Improving conditioning regimens for patients ahead of autologous transplant is a key focus for Beam as part of Wave 2 of our long-term strategy to bring best-in-class base editing treatments to people with SCD," said Giuseppe Ciaramella, Ph.D., president and chief scientific officer of Beam. "We are advancing our *ex vivo* programs with standard conditioning under our Wave 1 strategy and are excited by this Wave 2 progress with both our ESCAPE-1 and ESCAPE-2 approaches, which have the potential to expand the number of patients who may benefit from our therapies. These conditioning strategies take advantage of the strengths of base editing for precise, efficient, multiplex editing without double stranded breaks, and we are encouraged by the rapid progress we are making on our vision for a potentially non-genotoxic approach for conditioning in HSC transplant. This work is an integral part of our SCD portfolio strategy, and we believe it may also open many other potential applications in hematology. We look forward to presenting these data at the upcoming ASH Annual Meeting."

# **Presentation Details:**

**Title:** Engineered Stem Cell Antibody Paired Evasion 1 (ESCAPE-1): Paired HSC Epitope Engineering and Upregulation of Fetal Hemoglobin for Antibody-Mediated Autologous Hematopoietic Stem Cell Therapy Conditioning for the Treatment of Hemoglobinopathies (1955)

Session Name: 701. Experimental Transplantation: Basic and Translational: Poster I

Date & Time: Saturday, December 10, 2022, 5:30-7:30 p.m. Location: Ernest N. Morial Convention Center. Hall D

Title: Engineered Stem Cell Antibody Paired Evasion-2 (ESCAPE-2): Paired HSC Epitope Engineering and Direct Editing of Sickle Allele for Antibody-

Mediated Autologous Hematopoietic Stem Cell Therapy Conditioning for the Treatment of Sickle Cell Disease (4585)

Session Name: 701. Experimental Transplantation: Basic and Translational: Poster III

Date & Time: Monday, December 12, 2022, 6:00-8:00 p.m. Location: Ernest N. Morial Convention Center, Hall D

## **About Beam Therapeutics**

Beam Therapeutics (Nasdaq: BEAM) is a biotechnology company committed to establishing the leading, fully integrated platform for precision genetic medicines. To achieve this vision, Beam has assembled a platform that includes a suite of gene editing and delivery technologies and is in the process of building internal manufacturing capabilities. Beam's suite of gene editing technologies is anchored by base editing, a proprietary technology that is designed to enable precise, predictable and efficient single base changes, at targeted genomic sequences, without making double-stranded breaks in the DNA. This has the potential to enable a wide range of potential therapeutic editing strategies that Beam is using to advance a diversified portfolio of base editing programs. Beam is a values-driven organization committed to its people, cutting-edge science, and a vision of providing life-long cures to patients suffering from serious diseases.

## **Cautionary Note Regarding Forward-Looking Statements**

This press release contains forward-looking statements within the meaning of the Private Securities Litigation Reform Act of 1995. Investors are cautioned not to place undue reliance on these forward-looking statements, including, but not limited to, statements related to: our upcoming presentations at the ASH Annual Meeting and Exposition; the therapeutic applications and potential of our technology, including with respect to SCD and our conditioning regimens; and our ability to develop life-long, curative, precision genetic medicines for patients through base editing. Each forward-looking statement is subject to important risks and uncertainties that could cause actual results to differ materially from those expressed or implied in such statement, including, without limitation, risks and uncertainties related to: our ability to develop, obtain regulatory approval for, and commercialize our product candidates, which may take longer or cost more than planned; our ability to raise additional funding, which may not be available; our ability to obtain, maintain and enforce patent and other intellectual property protection for our product candidates; the potential impact of the COVID-19 pandemic, including its impact on the global supply chain; the uncertainty that our product candidates will receive regulatory approval

necessary to initiate human clinical studies; that preclinical testing of our product candidates and preliminary or interim data from preclinical studies and clinical trials may not be predictive of the results or success of ongoing or later clinical trials; that enrollment and initiation of our clinical trials may take longer than expected; that our product candidates may experience manufacturing or supply interruptions or failures; risks related to competitive products; and the other risks and uncertainties identified under the headings "Risk Factors Summary" and "Risk Factors" in our Annual Report on Form 10-K for the year ended December 31, 2021, and in any subsequent filings with the Securities and Exchange Commission. These forward-looking statements speak only as of the date of this press release. Factors or events that could cause our actual results to differ may emerge from time to time, and it is not possible for us to predict all of them. We undertake no obligation to update any forward-looking statement, whether as a result of new information, future developments or otherwise, except as may be required by applicable law.

This press release contains hyperlinks to information that is not deemed to be incorporated by reference in this press release.

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