Beam Therapeutics to Encore Data from BEACON Phase 1/2 Clinical Trial of BEAM-101 in Sickle Cell Disease at 2025 Tandem Meetings of ASTCT and CIBMTR

January 23, 2025

CAMBRIDGE, Mass., Jan. 23, 2025 (GLOBE NEWSWIRE) -- Beam Therapeutics Inc. (Nasdaq: BEAM), a biotechnology company developing precision genetic medicines through base editing, today announced the company will encore data from the BEACON Phase 1/2 clinical trial of BEAM-101 in sickle cell disease in an oral presentation at the 2025 Tandem Meetings | Transplantation & Cellular Therapy Meetings of the American Society for Transplantation and Cellular Therapy (ASTCT) and Center for International Blood and Marrow Transplant Research (CIBMTR) taking place February 12 – 15, 2025**,** in Honolulu, Hawaii.

Data from seven patients treated with investigational base-editing therapy BEAM-101 were previously presented at the 66th American Society of Hematology (ASH) Annual Meeting and Exposition in December 2024. Treatment with BEAM-101 demonstrated robust and durable increases in fetal hemoglobin (HbF) and reductions in sickle hemoglobin (HbS), rapid neutrophil and platelet engraftment, and normalized or improved markers of hemolysis. The safety profile of BEAM-101 was consistent with busulfan conditioning and autologous hematopoietic stem cell transplantation. Beam expects to present updated data from the BEACON trial in mid-2025.

Details for the oral presentation are as follows:

Title: Safety and Efficacy of Autologous CD34+ Base Edited Hematopoietic Stem Cells (BEAM-101) for the Treatment of Sickle Cell Disease with Severe Vaso-Occlusive Crises: Results from the Ongoing Phase 1/2 Beacon Study
Oral Session: Session M - Gene Therapy and Editing and Study Design and Statistics
Presentation Time: Wednesday, February 12, 2025, 3:15 p.m. HST
Presenter: Ashish Gupta, M.D., Ph.D., University of Minnesota

About BEAM-101
BEAM-101 is an investigational genetically modified cell therapy for the treatment of severe sickle cell disease (SCD). The one-time therapy consists of autologous CD34+ hematopoietic stem and progenitor cells (HSPCs) that have been base-edited in the promotor regions of the HBG1/2 genes and are administered via a hematopoietic stem cell transplant procedure. The BEAM-101 edit is designed to inhibit the transcriptional repressor BCL11A from binding to the promoter without disrupting BCL11A expression, leading to increased production of non-sickling and anti-sickling fetal hemoglobin (HbF) and thus mimicking the effects of naturally occurring variants seen in hereditary persistence of fetal hemoglobin. HbF is the predominant hemoglobin variant during development and early life. The safety and efficacy of BEAM-101 is being evaluated in the ongoing BEACON Phase 1/2 study, an open-label, single-arm, multicenter trial in adult patients with SCD with severe vaso-occlusive crises (VOCs).

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 with integrated gene editing, delivery and 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: the therapeutic applications and potential of our technology, including with respect to BEAM-101; our plans to advance our programs; 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 uncertainty that our product candidates will receive regulatory approval necessary to initiate human clinical trials; 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 initiation and enrollment of, and anticipated timing to advance, our clinical trials may take longer than expected; that our product candidates or the delivery modalities we rely on to administer them may cause serious adverse events; 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, 2023, our Quarterly Report on Form 10-Q for the quarter ended September 30, 2024, 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.

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