Beam Therapeutics to Present Updated Data from BEACON Phase 1/2 Trial of BEAM-101 in Sickle Cell Disease at American Society of Hematology (ASH) Annual Meeting

November 3, 2025

CAMBRIDGE, Mass., Nov. 03, 2025 (GLOBE NEWSWIRE) -- Beam Therapeutics Inc. (Nasdaq: BEAM), a biotechnology company developing precision genetic medicines through base editing, today announced that the company will present updated safety and efficacy data from the BEACON Phase 1/2 clinical trial of BEAM-101 in sickle cell disease (SCD) at the 67th American Society of Hematology (ASH) Annual Meeting and Exposition, taking place December 6-9, 2025, in Orlando. BEAM-101 is an investigational genetically modified *ex vivo* cell therapy for the treatment of SCD with severe vaso-occlusive crises (VOCs).

“Approximately eight million people worldwide live with sickle cell disease, a condition that can severely affect the function of multiple organs, diminish quality of life, and result in a shortened life span. These patients have a significant unmet need for additional safe and effective treatment options. BEAM-101, designed to be a one-time, disease-modifying therapy, has the potential to meaningfully improve outcomes for people with severe SCD,” said Amy Simon, M.D., chief medical officer of Beam. “We look forward to presenting updated data from the BEACON Phase 1/2 trial at the upcoming ASH Annual Meeting, which build on our earlier results and expand the body of evidence supporting BEAM-101 as a differentiated and potential best-in-class gene editing approach for SCD.”

Presentation details are as follows:

Title: Robust HbF Induction and Improvement of Anemia and Hemolysis with Base Editing in Sickle Cell Disease: Safety and Efficacy Findings from the Ongoing BEACON Study
Abstract: 2532
Poster Session: 801. Gene Therapies: Poster I.
Session Time: Saturday, December 6, 2025, 5:30 - 7:30 p.m. ET
Presenter: Ashish Gupta, M.D., MPH, University of Minnesota

Title: Enhanced CD34+ Cell Mobilizations, Collections, and Comparable Safety Profile with Fixed-Dose Versus Weight-based Plerixafor Dosing in Patients with Sickle Cell Disease Receiving Autologous CD34+ Base-edited Hematopoietic Stem Cells (BEAM-101) in the Ongoing BEACON study
Abstract: 1049
Oral Session: 711. Cell Collection and Manufacturing of HSPCs, CAR-T Cells, and Other Cellular Therapy Products: Refining CAR-T Cells and Engineered HSPCs; New Approaches to HSPC mobilization
Presentation Time: Monday, December 8, 2025, 5:30 – 5:45 p.m. ET
Presenter: Haydar Frangoul, M.D., Sarah Cannon Research Institute and TriStar Centennial

About BEAM-101
BEAM-101 is an investigational genetically modified cell therapy for the treatment of 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 promoter 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 Sickle Cell Disease
Sickle cell disease (SCD), a severe inherited blood disease, is caused by a single point mutation, E6V, in the beta globin gene. This mutation causes the mutated form of sickle hemoglobin (HbS) to aggregate into long, rigid molecules that bend red blood cells into a sickle shape under conditions of low oxygen. Sickled cells obstruct blood vessels and die prematurely, ultimately resulting in anemia, severe pain (crises), infections, stroke, organ failure and early death. SCD is the most common inherited blood disorder in the United States (U.S.), affecting an estimated 100,000 individuals within the U.S. and approximately eight million people worldwide.

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 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 SCD; our plans, and anticipated timing, to advance our programs, including the clinical trial designs and expectations for BEAM-101; our plans to present updated data at the 2025 ASH Annual Meeting; 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 advance 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, 2024, our Quarterly Reports on Form 10-Q, 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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