Beam Therapeutics Announces New Data from BEACON Phase 1/2 Clinical Trial of BEAM-101 Supporting Differentiated Profile in Sickle Cell Disease (SCD) at European Hematology Association (EHA) 2025 Congress

June 13, 2025

Updated Data from 17 Patients Consistent with Previously Presented Data; All Patients Treated with BEAM-101 Achieved Hemoglobin F (HbF) Induction of >60%, Hemoglobin S (HbS) Reduction to <40%, and Resolution of Anemia

Patients Required a Median of One Mobilization Cycle and Experienced Rapid Neutrophil and Platelet Engraftment

Safety Profile Remained Consistent with Busulfan Conditioning, Autologous Hematopoietic Stem Cell Transplantation and Underlying SCD

Enrollment Complete in Both Adult and Adolescent Cohorts of the BEACON Trial, with 30 Patients Expected to be Dosed by Mid-2025

Beam to Host Investor Webcast Today, June 13, 2025, at 4:00 p.m. ET

CAMBRIDGE, Mass., June 13, 2025 (GLOBE NEWSWIRE) -- Beam Therapeutics Inc. (Nasdaq: BEAM), a biotechnology company developing precision genetic medicines through base editing, announces new safety and efficacy data from its BEACON Phase 1/2 clinical trial of BEAM-101 in patients with sickle cell disease (SCD) with severe vaso-occlusive crises (VOCs). The data will be shared in a poster presentation today at the European Hematology Association 2025 Congress (EHA2025) in Milan.

New data from the BEACON trial with more patients and longer follow-up provide further demonstration of the strong clinical profile for the investigational base-editing therapy BEAM-101, as initially established in previously announced data at the 66th American Society of Hematology (ASH) Annual Meeting and Exposition in December 2024. Updated data from 17 patients treated 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 and oxygen delivery. Patients required a median of one mobilization cycle. No VOCs were reported post-engraftment. BEAM-101 is manufactured using an advanced, largely automated process that has demonstrated consistently high yields and viability, enabling successful manufacturing for all patients in the BEACON clinical trial as of the data cut-off. A summary of the results from the ongoing clinical study is provided below.

“There is significant demand for new, safe and effective treatments amongst patients living with SCD, many of whom grapple with severe symptoms that have a marked impact on their quality of life and lifespan,” said Ashish Gupta, M.D., MPH, pediatric blood and marrow transplant physician and associate professor at the University of Minnesota and an investigator in the BEACON trial. “These promising and consistent new data from the BEACON trial, now from 17 patients treated with BEAM-101, continue to reinforce the potential of this gene therapy to fulfill this unmet need. Patients only required a median of one mobilization cycle, had rapid neutrophil and platelet engraftment which minimized transfusion requirements, and achieved a stable hemoglobin F/S ratio that approximates sickle cell trait. Given this profile, BEAM-101 has the potential to further restore red blood cell health and function, reduce hospital time for patients, and decrease the overall burden of cell and gene therapy treatment for patients and providers.”

“We remain highly encouraged by the potential of BEAM-101, with today’s data further building on its potential to deliver a transformative treatment for patients with SCD,” said John Evans, chief executive officer of Beam. “We continue to see growing evidence of differentiated outcomes for BEAM-101 and base editing in severe SCD, now observed across 17 patients with the longest follow-up of over one year. Our manufacturing process has also delivered both flexibility for sites and patients as well as consistently strong product yields and success rates. With enrollment now complete in both the adult and adolescent cohorts, we are focused on continuing to dose patients in this trial as we work toward our mission of delivering life-long cures.”

As of a February 28, 2025, data cut-off, a total of 17 patients with severe SCD were treated with BEAM-101 and included in the safety and efficacy analysis, with follow-up ranging from 0.2 to 15.1 months.

Key highlights include the following:

- **Rapid and Sustained Increases in Protective HbF:** Consistent with data presented at ASH, all patients achieved endogenous HbF levels exceeding 60% and a durable reduction in corresponding HbS below 40%. A pancellular distribution of HbF-expressing cells, with HbF levels per cell above the sickling threshold, was maintained through follow-up.
- **Robust and Sustained Total Hemoglobin (Hb) Levels:** Total Hb levels increased rapidly with resolution of anemia in patients after elimination of the transfused blood.
- **Durable Responses Observed:** Increases in HbF, decreases in HbS and resolution of anemia were durable for up to 15 months.
- **Efficient Cell Collection and Rapid Engraftment:** Patients required a median of one mobilization cycle (range: 1-3 cycles). The median time to neutrophil engraftment was 16.5 days (range: 12-30), with a median duration of severe neutropenia of 7.0 days (range: 4-17). The median time to platelet engraftment was 19.5 days (range: 11-34).

- **Normalization of Hemolysis Markers and Erythropoietin Levels:** Key markers of hemolysis, including indirect bilirubin, haptoglobin, lactate dehydrogenase and reticulocytes, normalized or improved in all patients following BEAM-101 treatment. Erythropoietin levels also decreased to normal or near normal, indicating significant improvement in oxygen delivery to tissues.
- **Safety Profile Consistent with Busulfan and Autologous Hematopoietic Stem Cell Transplantation (HSCT):** The safety profile of BEAM-101 was consistent with busulfan conditioning, autologous HSCT and underlying SCD. The most common treatment-emergent adverse events (TEAEs) were consistent with busulfan conditioning, including stomatitis, febrile neutropenia and anemia. As previously reported, one patient died four months after BEAM-101 infusion due to respiratory failure that was determined by the investigator to be likely related to busulfan conditioning and deemed unrelated to BEAM-101.
- **No VOCs Reported**: No patients experienced any investigator-reported VOCs post-engraftment.
- **Improved Red Blood Cell (RBC) Health and Function**: Exploratory biomarker assessments of RBC health and function demonstrated improvements compared to baseline across multiple parameters after BEAM-101 treatment, including in multiple RBC sickling kinetic measurements to levels comparable to sickle cell trait, decreased RBC adhesion and percent dense RBCs, along with reduction in systemic inflammation.

Enrollment in the adult and adolescent cohorts of the BEACON trial is complete, and 26 patients were dosed with BEAM-101 as of June 13, 2025. Beam expects to dose 30 patients by mid-2025 and share additional data from the trial by the end of 2025.

EHA Investor Webcast Information
Beam will host a conference call and webcast today, June 13, 2025, at 4:00 p.m. ET to review the BEAM-101 data and other key presentations from this year's EHA meeting. A live webcast of the presentation will be available under "Events" in the Investors section of the company's website at www.beamtx.com, and a replay will be available shortly after the event.

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 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 BEAM-101 program, including the clinical trial designs and expectations for BEAM-101; 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 Report on Form 10-K for the quarter ended March 31, 2025, 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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