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

December 7, 2024

All Seven Patients Treated with BEAM-101 Achieved Hemoglobin F (HbF) Induction of >60%, Hemoglobin S (HbS) Reduction to <40%, and Resolution of Anemia Post-BEAM-101 Treatment

Initial Safety Profile Consistent with Busulfan Conditioning and Autologous Hematopoietic Stem Cell Transplantation

All Seven Patients Dosed Achieved Target Cell Dose with One or Two Mobilization Cycles and Experienced Rapid Neutrophil and Platelet Engraftment

Markers of Hemolysis Normalized or Improved in All Patients

Beam to Host Investor Event on Dec. 8, 2024, at 8 p.m. PT

SAN DIEGO, Dec. 07, 2024 (GLOBE NEWSWIRE) -- Beam Therapeutics Inc. (Nasdaq: BEAM), a biotechnology company developing precision genetic medicines through base editing, today announced 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 were featured today in the press program for the 66th American Society of Hematology (ASH) Annual Meeting and Exposition in San Diego and will be presented in an oral session on Sunday, Dec. 8, 2024, at 10 a.m. PT.

Consistent with Beam's previously announced data, updated data from seven patients treated with investigational base-editing therapy 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. No VOCs were reported post-engraftment. A summary of the results from the ongoing clinical study is provided below.

"These initial data from the BEACON trial are very encouraging and highlight the potential of BEAM-101 to deliver meaningful clinical benefits to patients with severe sickle cell disease," said Matthew M. Heeney, M.D., associate chief of hematology at Dana-Farber/Boston Children's Cancer and Blood Disorders Center. "The data from the first seven patients demonstrate the ability for BEAM-101 to dramatically modify the hemoglobin profile to express a majority of protective fetal hemoglobin. All patients mobilized efficiently and had rapid engraftment with a low number of neutropenic days. I look forward to the continued maturation of the data to provide further insights into the long-term benefits of BEAM-101 for people living with sickle cell disease."

"It's an honor to share the initial results from BEACON with the hematology community at the ASH Annual Meeting, where there is broad recognition of the significant burden that sickle cell disease places on patients and their families," said John Evans, chief executive officer of Beam. "We believe these early data for BEAM-101 are a testament to the potential of our base-editing technology to provide a differentiated option for sickle cell patients, having demonstrated a robust increase in fetal hemoglobin of >60%, a decrease in hemoglobin S to <40% and resolution of anemia in all patients. Additionally, the data from our ESCAPE nongenotoxic conditioning program – to be presented on Sunday – highlight our commitment to expanding access to treatment by decreasing the burden and complications patients potentially face when undergoing transplantation. We look forward to continuing to rapidly advance both programs for patients with sickle cell disease."

To date, more than 35 patients have cleared screening and enrolled in the BEACON Phase 1/2 clinical trial, and of these, 11 patients have been dosed with BEAM-101. As of an Oct. 28, 2024, data cut-off, a total of seven patients with severe SCD were treated with BEAM-101 and included in the safety and efficacy analysis with follow up ranging from 1 to 11 months.

Key highlights include the following:

- **Rapid and Sustained Increases in Protective Fetal Hemoglobin (HbF)**: All patients achieved endogenous HbF levels exceeding 60% and reduction in corresponding sickle hemoglobin (HbS) below 40% that was durable. A pancellular distribution of HbF was observed after the elimination of transfused blood.
- **Robust and Sustained Total Hemoglobin (Hb) Levels**: Total hemoglobin levels increased rapidly with resolution of anemia in patients after elimination of the transfused blood.
- **Efficient Cell Collection and Rapid Engraftment**: All patients achieved the minimum target cell dose in either 1 or 2 cycles of mobilization (average: 1.4). The mean time to neutrophil engraftment was 17.1 days (range: 15–21), with a low mean duration of neutropenia (6.3 days). The mean time to platelet engraftment was 19.1 days (range: 11–34).
- **Normalization of Hemolysis Markers**: Key markers of hemolysis, including indirect bilirubin, haptoglobin, lactate dehydrogenase and reticulocytes, normalized or improved in all patients following BEAM-101 treatment.
- **Safety Profile Consistent with Busulfan and Autologous Hematopoietic Stem Cell Transplantation (HSCT)**: The safety profile of BEAM-101 was consistent with busulfan conditioning and autologous HSCT. The most common treatment-emergent adverse events (TEAEs) were consistent with busulfan conditioning, including febrile neutropenia, stomatitis and anemia. 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 were reported

post-engraftment.

ASH Investor Event Information
Beam will host a live and webcast investor event on Dec. 8, 2024, at 8:00 p.m. PT in San Diego to review the key presentations from this year's ASH meeting. The event will be webcast live and can be accessed under "Events & Presentations" in the Investors section of the company's website at www.beamtx.com. The archived webcast will be available on the company's website beginning approximately two hours 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 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 SCD; our plans, and anticipated timing, to advance our programs; the clinical trial designs and expectations for BEAM-101 and ESCAPE; our presentations at the 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 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 Reports on Form 10-Q for the quarterly period 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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