# Beam Therapeutics to Highlight New Data from BEAM-101 Program in Sickle Cell Disease at European Hematology Association (EHA) 2025 Congress

May 14, 2025

*Presentation to Include Updated Data from 17 Sickle Cell Disease Patients in the Ongoing BEACON Phase 1/2 Clinical Trial Evaluating Safety and Efficacy of BEAM-101*

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

CAMBRIDGE, Mass., May 14, 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 new data from the BEACON Phase 1/2 clinical trial of BEAM-101 in sickle cell disease (SCD) at the European Hematology Association 2025 Congress (EHA2025), taking place June 12-15, 2025, in Milan, Italy. BEAM-101 is an investigational genetically modified *ex vivo* cell therapy for the treatment SCD with severe vaso-occlusive crises (VOCs).

“We are excited to share an updated safety and efficacy dataset from 17 patients in the BEACON Phase 1/2 clinical trial at EHA2025, as we continue to build on our understanding of this potentially transformative, one-time treatment for people living with SCD,” said Amy Simon, M.D., chief medical officer of Beam. “SCD affects millions of people worldwide, and we are committed to delivering a potential lifelong treatment that addresses the root cause of this debilitating condition. These new data, along with additional insights from our other presentations at EHA2025, further enhance our understanding of the disease, support a differentiated profile of BEAM-101 and underscore the promise of base editing in creating precision therapies for patients.”

Presentation details are as follows:

**BEAM-101 Presentations:**

**Title:** Base Editing for Sickle Cell Disease: Ongoing Results from the BEACON Study Evaluating the Safety and Efficacy of BEAM-101, the First Base-Edited Autologous CD34+ HSPC One-Time Cell Therapy
**Abstract:** PF1151
**Poster Session:** Poster Session 1
**Session Time:** Friday, June 13, 2025, 6:30 - 7:30 p.m. CEST
**Presenter:** Ashish Gupta, M.D., MPH, University of Minnesota

**Title:** Red Blood Cell (RBC) Health and Function Post BEAM-101 Treatment: Multiple Exploratory Biomarkers Demonstrate Rheology and Sickling Parameters Comparable to Sickle Cell Trait (SCT)
**Abstract:** PF1155
**Poster Session:** Poster Session 1
**Session Time:** Friday, June 13, 2025, 6:30 - 7:30 p.m. CEST
**Presenter:** Priya S. Chockalingam, Ph.D., Beam Therapeutics

**Title:** Integrated Editing and Manufacturing Process Design for BEAM-101, an Autologous CD34+ HSPC Therapy for Sickle Cell Disease, Results in Robust Process Yield and Increased HbF Induction
**Abstract:** PF1165
**Poster Session:** Poster Session 1
**Session Time:** Friday, June 13, 2025, 6:30 - 7:30 p.m. CEST
**Presenter:** Paul Kopesky, Ph.D., Beam Therapeutics

**Collaborator Presentation:**

**Title:** Identifying Potential Biomarkers of Clinical Relevance in Sickle Cell Trait: Interim Findings from the National Alliance of Sickle Cell Centers AUNT Study
**Abstract:** PF1174
**Poster Session:** Poster Session 1
**Session Time:** Friday, June 13, 2025, 6:30 - 7:30 p.m. CEST
**Presenter:** Julie Kanter, M.D., the University of Alabama at Birmingham

**EHA Investor Webcast Information**
Beam will host a conference call and webcast on Friday, June 13, 2025, at 4:00 p.m. ET to review 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 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 Sickle Cell Disease (SCD)**
Sickle cell disease, 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. Sickle cell disease is the most common inherited blood disorder in the United States, affecting an estimated 100,000 individuals within the United States 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; our plans to present data at EHA2025; 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.

**Contacts:**

Investors:
Holly Manning
Beam Therapeutics
hmanning@beamtx.com

Media:
Josie Butler
1AB
josie@1abmedia.com