Beam Therapeutics to Present Updated Clinical Data from BEACON Phase 1/2 Trial of Risto-cel in Sickle Cell Disease in an Oral Presentation at American Society of Hematology (ASH) Annual Meeting

November 4, 2026

BEACON Trial Results Selected for Official ASH Press Program

Initial Patient-Reported Outcomes Demonstrate Early and Sustained Improvements in Physical Function, Pain, Fatigue, Emotional and Social Well-Being and Symptom Severity

U.S. Biologics License Application (BLA) Submission for Risto-cel Expected as Early as Year-End 2026

CAMBRIDGE, Mass., Nov. 04, 2026 (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 data from the BEACON Phase 1/2 clinical trial of ristoglogene autogetemcel (risto-cel) in sickle cell disease (SCD) in an oral presentation at the 68th American Society of Hematology (ASH) Annual Meeting and Exposition, taking place December 12–15, 2026, in New Orleans. The presentation has also been selected for ASH's official press program. Risto-cel is an investigational autologous genetically modified cell therapy being evaluated for the treatment of SCD.

"With more patients and longer follow-up, the updated BEACON data continue to demonstrate risto-cel's differentiated clinical and manufacturing profile and its potential to provide a meaningful treatment option for patients living with sickle cell disease," said Amy Simon, M.D., chief medical officer of Beam. "At ASH, we look forward to sharing updated findings from a growing population of adolescent and adult patients from the trial, including, initial patient-reported outcomes data demonstrating improvements compared to baseline across measures of physical function, pain, fatigue and overall well-being. These findings reinforce our confidence in risto-cel as we prepare for an anticipated BLA submission as early as year-end 2026 and work toward bringing this potentially transformative treatment to patients."

Presentation details are as follows:

Title: Efficacy, Safety, and Patient-Reported Outcomes of Risto-cel in Adolescents and Adults with Sickle Cell Disease: Interim Results from the BEACON Study
Abstract: 335
Oral Session: 114. Sickle Cell Disease, Sickle Cell Trait, and Other Hemoglobinopathies, Excluding Thalassemias: Clinical and Epidemiological - Therapeutics
Presentation Time: Saturday, December 12, 2026, 5:00–5:15 p.m. CT
Presenter: Ashish Gupta, M.D., MPH, University of Minnesota

Additional abstract accepted for publication only:

Title: Fetal Hemoglobin as a Predictor of Clinical Outcomes and Sickling Parameters in Sickle Cell Disease: A Model-Based Meta-Analysis
Format: Publication-only in the online abstract issue of *Blood,* ASH's official journal
Author: Haydar Frangoul, M.D., Sarah Cannon Research Institute and TriStar Centennial

About ristoglogene autogetemcel (risto-cel, formerly known as BEAM-101)
Risto-cel 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 risto-cel 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 risto-cel are being evaluated in the ongoing BEACON Phase 1/2 study, an open-label, single-arm, multicenter trial in 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 lifelong 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 base editing technology and risto-cel; the potential of risto-cel to provide a meaningful treatment option for patients with sickle cell disease; the significance of the updated clinical data from the BEACON Phase 1/2 trial, including patient-reported outcomes data; our expected presentation at the ASH Annual Meeting; our plans to advance risto-cel, including our anticipated BLA submission as early as year-end 2026, and our other programs; the sufficiency of our capital resources to fund operating expenses and capital expenditure requirements; and our ability to develop lifelong, 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 submit a BLA for risto-cel on the timing we anticipate; 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 could delay, limit or prevent further clinical development; that our product candidates may experience manufacturing or supply interruptions or failures; risks related to competitive products; our ability to build and scale commercial infrastructure, including a sales and marketing organization, to support potential product launches; 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, 2025, our Quarterly Report on Form 10-Q for the quarter ended September 30, 2026, 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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