Beam Therapeutics Announces Publication of BEACON Phase 1/2 Data for risto-cel in Patients with Sickle Cell Disease (SCD) in The New England Journal of Medicine

April 1, 2026

Prestigious Publication Reinforces risto-cel's Durable, Differentiated Clinical Data for the Treatment of SCD with Severe Vaso-occlusive Crises (VOCs)

Initially Presented at ASH, Data from 31 Patients with SCD Demonstrated Deep Resolution of Red Blood Cell Dysfunction and Reduced Time in Hospital Driven by a Median of One Cell Collection Cycle and Rapid Engraftment

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

CAMBRIDGE, Mass., April 01, 2026 (GLOBE NEWSWIRE) -- Beam Therapeutics Inc. (Nasdaq: BEAM), a biotechnology company developing precision genetic medicines through base editing, today announced the publication of data from the ongoing Phase 1/2 BEACON clinical trial evaluating ristoglogene autogetemcel (risto-cel, formerly known as BEAM-101) for the treatment of sickle cell disease (SCD) with severe vaso-occlusive crises (VOCs) in *The New England Journal of Medicine* (NEJM). Risto-cel is an investigational autologous cell therapy with a potential best-in-class profile for the treatment of SCD.

"The publication of these interim data from the BEACON trial in *The New England Journal of Medicine* underscores the potential of risto-cel to make a transformational difference in the lives of patients living with SCD by reducing severe pain crises and the progressive complications that impact quality of life and lifespan," said Matthew M. Heeney, M.D., the associate chief of hematology at the Dana-Farber/Boston Children's Cancer and Blood Disorders Center and corresponding author of the NEJM publication. "In the data presented, risto-cel demonstrated an acceptable safety profile consistent with myeloablative conditioning, as well as very encouraging efficacy with rapid engraftment and induction of non-sickling fetal hemoglobin to levels sustained above 60% with concomitant reduction of sickle hemoglobin to below 40%, achieving a protective hemoglobin ratio similar to sickle cell trait. These changes resulted in improved hemolysis parameters, resolution of anemia, and no reported severe VOCs following engraftment. Risto-cel has the potential to meaningfully alter the pathophysiology of the disease and improve outcomes for patients with SCD."

The data included in the NEJM publication were most recently presented at the 67th American Society of Hematology (ASH) Annual Meeting and Exposition and continue to show evidence of risto-cel's differentiated treatment profile. As of the August 6, 2025, data cut-off, a total of 31 patients with SCD with severe VOCs were treated with risto-cel in the BEACON Phase 1/2 trial and are included in the safety and efficacy analysis. Follow-up ranged from 0.3 to 20.4 months. Risto-cel's efficient cell collection and manufacturing processes, combined with high, predictable yields from base editing, resulted in patients requiring a median of one stem cell collection cycle to manufacture risto-cel. The median time from start of cell collection to drug product release was 2.9 months, and the median time from start of cell collection to dosing was 4.5 months. Patients achieved rapid and robust bone marrow reconstitution post-risto-cel treatment, and no patients experienced any investigator-reported severe VOCs post-engraftment. Patients achieved mean hemoglobin F (HbF) levels above 60% and a mean durable reduction in corresponding hemoglobin S (HbS) below 40%. Total Hb levels increased rapidly with all patients experiencing resolution of anemia after elimination of the transfused blood, and key markers of hemolysis normalized or improved in all patients following risto-cel treatment. Sickling parameters all decreased in the blood following risto-cel treatment to levels comparable to those seen in individuals with sickle cell trait. The safety profile of risto-cel was consistent with busulfan conditioning, autologous hematopoietic stem cell transplantation (HSCT) and underlying SCD.

"In addition to the compelling safety and efficacy data, it is encouraging to see the often arduous and challenging operational aspects of the cell collection, manufacturing and transplant process trending favorably and having consistency across multiple centers," said Ashish Gupta, M.D., MPH, associate professor, pediatric blood and marrow transplant and cellular therapies at the University of Minnesota, and lead author of the NEJM publication. "With few cell collection cycles, rapid engraftment and reduced transfusion needs, risto-cel has the potential to improve the treatment experience for both patients as well as reduce resource utilization at treating centers."

"Risto-cel reflects our vision for how base editing can enable a new class of precision genetic medicines designed to deliver robust and durable benefits for patients," said Amy Simon, M.D., chief medical officer of Beam. "Based on the totality of data generated to date from the BEACON study and the optimized treatment and manufacturing process, we believe risto-cel has the potential to be best-in-class. With an estimated 10,000 patients with SCD in the U.S. eligible for gene therapies, and significant demand expected as these treatments become more widely available, risto-cel is well positioned within this growing market. We are focused on advancing risto-cel toward a planned BLA submission as early as year-end 2026 and bringing this therapy to patients as quickly as possible."

The adult and adolescent cohorts of the BEACON trial were fully enrolled in mid-2025 and manufacturing of all doses was completed as of December 2025. The FDA previously granted risto-cel orphan drug designation and Regenerative Medicine Advanced Therapy (RMAT) designation. Risto-cel has also been accepted into the FDA's Chemistry, Manufacturing, and Controls Development and Readiness Pilot (CDRP) program.

The full publication, "Base Editing of HBG1 and HBG2 Promoters for Sickle Cell Disease," appears in the April 1, 2026, issue of *The New England Journal of Medicine*.

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 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 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 technology, including with respect to SCD; our plans, and anticipated timing, to advance our risto-cel program; 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 or continue 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, including 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, 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.

Disclosure Statement: Matthew M. Heeney, M.D., and Ashish Gupta, M.D., MPH, consult for Beam Therapeutics.

Contacts:
Investors:
Holly Manning
Beam Therapeutics
hmanning@beamtx.com

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