



Beam Therapeutics to Present Initial Data from Phase 1/2 Trial of BEAM-301 in Glycogen Storage Disease Type Ia (GSDIa) at the ESGCT 33rd Annual Congress

October 13, 2026

BEAM-301 Achieves Clinical Proof of Concept for Genetic Correction of R83C Mutation in GSDIa

Initial Phase 1/2 Data Show Restoration of Endogenous Glucose Production Following a Single Treatment, with Dose-Dependent Improvements in Key GSDIa Endpoints

Safety Profile of BEAM-301 Consistent with LNP-based Therapies

Beam to Host Investor Webcast on Tuesday, October 27, 2026, at 8:00 a.m. ET

CAMBRIDGE, Mass., Oct. 13, 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 initial data from the ongoing Phase 1/2 clinical trial evaluating BEAM-301 for the treatment of glycogen storage disease type Ia (GSDIa) at the 33rd Annual Congress of the European Society of Gene and Cell Therapy (ESGCT), taking place October 27-30, 2026, in Hamburg, Germany. BEAM-301 is a liver-targeting lipid-nanoparticle (LNP) formulation of base editing reagents designed to correct the R83C mutation, one of the most prevalent disease-causing mutations in GSDIa and one that is associated with the most severe form of the disease.

"People living with GSDIa face a relentless daily burden, including dependence on frequent starch administration, even throughout the night, to maintain glucose levels and prevent life-threatening hypoglycemia," said Amy Simon, M.D., chief medical officer of Beam. "These initial data provide the first clinical evidence suggesting that BEAM-301, a base editing therapy designed to directly correct an underlying disease-causing mutation in patients with GSDIa, restored endogenous glucose production following a single *in vivo* treatment. Most notably, evaluable patients in the 40 mg cohort achieved ≥ 15 hours of controlled fasting and experienced substantial reductions in starch requirements. While follow-up remains ongoing, these findings support the transformative potential of BEAM-301 and represent an important proof of concept that precision genetic medicines, customized to a patient's specific mutation, can address the root cause of their disease."

Details are as follows:

Title: Initial Data from the Phase 1/2 Clinical Study of BEAM-301, an *In Vivo* Base Editor Targeting the R83C Variant in Glycogen Storage Disease Type Ia (GSDIa)

Poster ID: P0219

Presentation Session: Poster Session I

Presentation Session Time: Wednesday, October 28, 1:30–3:30 p.m. CET

Presenter: Rebecca Riba-Wolman, M.D., Connecticut Children's Hospital

Key Highlights:

- As of the June 26, 2026, data cutoff, six patients received a single intravenous infusion of BEAM-301, including three patients treated with 20 mg and three patients treated with 40 mg. Median follow-up was 313 days (range, 249–453) in the 20 mg cohort and 53 days (range, 5–110) in the 40 mg cohort. One patient in the 40 mg cohort was not evaluable for efficacy as of the data cutoff.
- BEAM-301 demonstrated an acceptable safety profile that was consistent with LNP-based therapies. All patients experienced at least one treatment-emergent adverse event considered related to BEAM-301. Five serious adverse events were reported and resolved within two to 10 days, including three Grade 3 infusion-related reactions (IRRs). The use of shorter-acting steroids as premedication, which is typical in this patient population, may have contributed to the Grade 3 IRRs observed. The steroid premedication regimen has since been optimized. All transaminase elevations were transient and resolved without intervention. With the exception of a single Grade 2 elevation in aspartate aminotransferase (AST), all transaminase elevations were Grade 1. No dose-limiting toxicities, treatment discontinuations due to treatment-emergent adverse events, or deaths were reported.
- Dose-dependent improvements in time to fasting hypoglycemia (TTH) were observed. At baseline, median TTH was 2.9 hours in the 20 mg cohort and 2.5 hours in the 40 mg cohort. By Day 36, median TTH increased to 4.3 hours in the 20 mg cohort, while both evaluable patients in the 40 mg cohort achieved the protocol-defined maximum controlled fasting duration of ≥ 15 hours, consistent with restored endogenous glucose production.
- Reductions in prescribed daily starch intake were observed following treatment with BEAM-301. Decreases from baseline in prescribed daily starch intake guided by continuous glucose monitoring were 74% (Day 128), 71% (Day 227), and 11% (Day 387) in the 20 mg cohort, and 71% (Day 50) and 92% (Day 93) in the 40 mg cohort. Corresponding daily starch doses decreased by 0–4 and 2–4 doses/day in the 20 mg cohort and the 40 mg cohort (baseline: 6–7 and 5–6 doses/day), respectively. Triglyceride levels were comparable to baseline at last follow-up.
- Updated data from the ongoing Phase 1/2 trial will be presented during the ESGCT poster session.

ESGCT Investor Event Information

Beam will host a live and webcast investor event on Tuesday, October 27, at 8:00 a.m. ET to review the data presented at this year's ESGCT congress. 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-301

BEAM-301 is a liver-targeting lipid-nanoparticle (LNP) formulation of base editing reagents designed to correct the R83C mutation, one of the most prevalent disease-causing mutations and one that results in the most severe form of glycogen storage disease type Ia (GSDIa). BEAM-301 is being evaluated in a Phase 1/2, open-label, dose-exploration and dose-expansion clinical trial to investigate its safety, tolerability, pharmacodynamics, pharmacokinetics and efficacy in patients with GSDIa.

About Glycogen Storage Disease Type Ia (GSDIa)

GSDIa is an autosomal recessive disorder caused by mutations in the G6PC1 gene that disrupt a key enzyme, glucose-6-phosphatase (G6Pase), critical for maintaining glucose homeostasis. Inhibition of G6Pase activity results in low fasting blood glucose levels that can result in seizures and death. Patients with the G6PC1 R83C mutation typically require frequent cornstarch administration, without which they may enter into hypoglycemic shock within one to three hours. There remains significant unmet need for patients with GSDIa.

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 BEAM-301; the potential of BEAM-301 to restore endogenous glucose production and reduce starch requirements in patients with GSDIa; the significance of the initial clinical data as proof of concept for precision genetic medicines; our expected presentation at the ESGCT conference; our plans to advance BEAM-301 and our other programs; the sufficiency of our capital resources to fund operating expenses and capital expenditure requirements; 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 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 June 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.

Contacts:

Investors:

Holly Manning

Beam Therapeutics

hmanning@beamtx.com

Media:

Josie Butler

1AB

josie@1abmedia.com