



Beam Therapeutics Reports Initial Clinical Data from Phase 1/2 Trial of BEAM-301, Demonstrating Proof of Concept for Genetic Correction in Glycogen Storage Disease Type Ia (GSDIa) at ESGCT 33rd Annual Congress

October 27, 2026

In the 40 mg Cohort, Two Patients Achieved 15 Hours of Controlled Fasting, Improvements in Glucose Control and No Longer Required Starch Supplementation Following Treatment

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

Beam Plans to Engage FDA on Potential Path to Approval; Enrollment in 60 mg Dose Cohort Underway

Company to Host Investor Webcast Today, October 27, 2026, at 8:00 a.m. ET

CAMBRIDGE, Mass., Oct. 27, 2026 (GLOBE NEWSWIRE) -- Beam Therapeutics Inc. (Nasdaq: BEAM), a biotechnology company developing precision genetic medicines through base editing, today reported initial data from the ongoing Phase 1/2 clinical trial evaluating BEAM-301 for the treatment of glycogen storage disease type Ia (GSDIa). The data will be presented during a poster session at the 33rd Annual Congress of the European Society of Gene and Cell Therapy (ESGCT) in Hamburg, Germany. BEAM-301 is an investigational 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.

"These findings demonstrate the potential for BEAM-301 to fundamentally change how patients with GSDIa manage their disease," said Rebecca Riba-Wolman, M.D., director of the glycogen storage disease program and disorders of hypoglycemia at Connecticut Children's and University of Connecticut School of Medicine, and presenting investigator in the BEAM-301 Phase 1/2 trial. "The ability of two patients to achieve 15 hours of controlled fasting and no longer require starch supplementation following a single treatment represents a significant departure from the intensive daily management typically required for this disease. While these are early results, the improvements in glucose regulation and reductions in starch requirements provide encouraging evidence of BEAM-301's potential to deliver meaningful and lasting benefits for patients."

BEAM-301 Phase 1/2 Clinical Trial Data Update

BEAM-301 is being evaluated in an open-label Phase 1/2 dose-exploration trial to investigate its safety, tolerability, pharmacodynamics, pharmacokinetics and efficacy in patients with GSDIa who are homozygous or compound heterozygous for the R83C variant. As of the August 17, 2026, data cutoff, six patients had 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 across both cohorts was 7.6 months (range: 1.9–16.6 months).

Key highlights include:

- **Safety Profile Consistent with LNP-Based Therapies:** BEAM-301 demonstrated an acceptable safety profile consistent with LNP-based therapies, with no dose-limiting toxicities, Grade 4 or 5 treatment-emergent adverse events (TEAEs), or treatment discontinuations due to TEAEs reported. All patients experienced at least one TEAE considered related to BEAM-301. Four of six patients experienced serious TEAEs considered related to BEAM-301, including three Grade 3 infusion-related reactions (IRRs). Premedication with shorter-acting steroids, which are typically used in this patient population, may have contributed to the Grade 3 IRRs observed. The steroid premedication and infusion rate regimens have since been modified. Two other serious TEAEs of diarrhea and rhabdomyolysis occurred in two patients; both have resolved. With the exception of a single Grade 2 elevation in aspartate aminotransferase (AST), all transaminase elevations were Grade 1. All liver enzyme elevations resolved without treatment, and total bilirubin remained within normal limits.
- **Restoration of Endogenous Glucose Production Following a Single Treatment:** BEAM-301 demonstrated dose-dependent improvements in time to fasting hypoglycemia (TTH), a key measure of glucose regulation in patients with GSDIa. Median TTH increased from 2.9 hours at baseline to 4.3 hours at Day 36 in the 20 mg cohort, and from 2.5 hours at baseline to 15.0 hours at Day 36 in the 40 mg cohort. Two of three patients in the 40 mg cohort achieved the protocol-defined maximum controlled fasting duration of 15 hours, consistent with restored endogenous glucose production. During the 15-hour fasting challenges, blood glucose levels were generally maintained within the euglycemic range. One patient in the 40 mg cohort did not show an increase in TTH at the first post-dose assessment; several factors may have contributed, including elevated body mass index (BMI) and heterozygosity for the R83C mutation.
- **100% Reduction in Daily Starch Supplementation Observed in Two Patients:** Daily starch intake and daily number of starch doses generally decreased following treatment with BEAM-301 (baseline: 6–7 and 5–6 doses/day in the 20 mg and 40 mg cohorts, respectively), with two of three patients in the 40 mg cohort achieving a 100% reduction in daily starch requirements and no longer requiring starch supplementation at their latest reported assessments.
- **Improvements in Glucose Control Despite Reduced Starch Intake:** Continuous glucose monitoring demonstrated

increased time spent in the euglycemic range and decreased time in the hyperglycemic range following treatment with BEAM-301, despite reductions in daily starch intake. Patients generally did not experience an increase in time spent in hypoglycemia. Triglyceride levels were generally comparable to baseline at the latest evaluable time point.

Based on the safety and clinical findings observed to date, Beam is advancing BEAM-301 into the 60 mg dose-escalation cohort, with enrollment currently underway. Beam also plans to engage with the U.S. Food and Drug Administration (FDA) regarding a potential path to approval.

"The promise of base editing is to deliver transformative outcomes that correct the root cause of disease with a one-time therapy. These initial results show that we are continuing to advance toward that vision, generating important clinical proof of concept for BEAM-301 in GSDIa and once again demonstrating the potential of base editing to fundamentally change the treatment paradigm for patients," said John Evans, chief executive officer of Beam Therapeutics. "These findings also reinforce the strength of our fully integrated platform, combining our proprietary base editing technology, validated delivery capabilities, and internal manufacturing to enable the predictable development and rapid production of precision genetic medicines. We look forward to building on these encouraging results as we advance BEAM-301 into the next dose cohort, engage with regulators on a potential pathway to approval, and progress our broader pipeline."

ESGCT Investor Event Information

Beam will host a live and webcast investor event today, 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 an investigational 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 engage with the FDA regarding a potential path to approval for BEAM-301; 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 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 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.

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