Beam Therapeutics Announces Compelling Updated Clinical Data from the Ongoing Phase 1/2 Trial of BEAM-302 in Alpha-1 Antitrypsin Deficiency (AATD) to Support Advancement to Pivotal Development

March 25, 2026

Treatment with 60 mg of BEAM-302 Led to Mean Steady-state Total AAT Level of 16.1 µM and All Patients Consistently and Durably Above the 11 µM Protective AAT Threshold with up to 12 Months of Follow-up

Corrected M-AAT Comprised 94% of Total AAT with a Concomitant 84% Reduction in Mutant Z-AAT Following BEAM-302 60 mg Treatment

Post-treatment Inducibility of AAT Observed During Respiratory Infection with a Patient Reaching ~30 µM Total AAT, Retaining 95% M-AAT Composition

Well-tolerated Safety Profile Observed with Single Doses of BEAM-302 up to 75 mg; Safety Consistent Across Single-dose Part A and Part B Cohorts

60 mg Selected as Optimal Biological Dose, Supported by Strong Safety and Efficacy Profile Across Single-dose Cohorts; Global Pivotal Cohort Expected to Initiate in Second Half of 2026

Beam to Host Investor Webcast Today, March 25, 2026, at 8:00 a.m. ET

CAMBRIDGE, Mass., March 25, 2026 (GLOBE NEWSWIRE) -- Beam Therapeutics Inc. (Nasdaq: BEAM), a biotechnology company developing precision genetic medicines through base editing, today announced updated safety and efficacy data from the ongoing Phase 1/2 trial of BEAM-302 and the selection of 60 mg as the optimal biological dose to advance into pivotal development to support potential accelerated approval. BEAM-302 is a liver-targeting lipid-nanoparticle (LNP) formulation designed to directly correct the underlying genetic mutation that causes the severe form of alpha-1 antitrypsin deficiency (AATD) through base editing.

“AATD is a serious genetic disease that can lead to significant liver disease over an individual’s lifespan along with progressive lung disease in adults, often leaving patients with limited treatment options and challenging, lifelong disease management,” said Jeffrey Teckman, M.D., professor of pediatrics, Saint Louis University School of Medicine. “What makes BEAM-302 particularly compelling is its ability to directly correct the underlying genetic mutation in the *SERPINA1* gene that drives both lung and liver manifestations of the disease. By enabling the liver to produce corrected M-AAT for the first time while reducing the toxic mutant protein, this approach has the potential to fundamentally transform how we as clinicians treat AATD and represents a meaningful advance for patients.”

BEAM-302 Phase 1/2 Clinical Trial Data Update
BEAM-302 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. Part A of the trial is designed to evaluate patients with AATD-associated lung disease, while Part B evaluates patients with mild to severe liver disease, with or without lung disease. As of the February 10, 2026 data cutoff date, 29 patients have been treated with BEAM-302 in Part A (15 mg, n=3; 30 mg, n=3; 60 mg, n=6; 75 mg, n=9; 2x 60 mg, n=3) and Part B (30 mg, n=3; 60 mg, n=2) and followed for up to 18 months.

Data from 26 patients treated with single-dose BEAM-302 support a well-tolerated safety profile up to 75 mg that is consistent across Part A and Part B. Adverse events (AEs) were mild to moderate, with no serious AEs reported and no dose-limiting toxicities as of the data cutoff. Transient Grade 1 and Grade 2 infusion-related reactions (IRRs) and Grade 1 asymptomatic alanine transaminase (ALT) and aspartate aminotransferase (AST) elevations were observed. In the multi-dose cohort (n=3), following the second dose of BEAM-302, patients experienced Grade 2 IRRs, one patient had Grade 4 ALT and Grade 3 AST elevations, and one patient had a Grade 2 ALT elevation. All ALT/AST elevations were asymptomatic and did not require treatment. No bilirubin increases were observed in any patient.

Treatment with BEAM-302 led to rapid and durable increases of total and functional AAT, decreases in mutant Z-AAT, and new production of corrected M-AAT. Key data from 28 efficacy evaluable patients[1] include the following:

- After treatment with a single dose of BEAM-302 in Part A, the steady-state[2] circulating total AAT mean (LC-MS)[3] was 16.1 µM in the 60 mg cohort (follow-up ranging from 5-12 months) and 14.4 µM in the 75 mg cohort (follow-up ranging from 2-9 months). In the multi-dose cohort, patients achieved a mean of 16.5 µM total AAT at Day 84, 28 days after the second 60 mg dose. These early data suggest a single dose of 60 mg BEAM-302 has achieved near saturation editing.
- Across all cohorts, increased total AAT in circulation was functional as demonstrated by a neutrophil elastase inhibition assay.
- Mutant Z-AAT was durably and significantly reduced after treatment with BEAM-302. The steady-state mean reduction in Z-AAT was 84% in the 60 mg cohort and 79% in the 75 mg cohort. In the multi-dose cohort, the mean reduction in Z-AAT was 80% at Day 84.
- Evidence of dynamic induction of AAT expression was observed during a respiratory infection around Month 8 in a patient in the 60 mg Part A cohort. During the infection, total AAT levels increased from steady-state levels of 15.9 µM to 29.5 µM while maintaining consistent AAT composition of 95% M-AAT.

- Following treatment with BEAM-302, newly produced corrected M-AAT comprised the majority of AAT in circulation. The steady-state mean proportion of M-AAT was 94% in the 60 mg cohort and 91% in the 75 mg cohort. In the multi-dose cohort, the mean proportion of M-AAT was 93% at Day 84.
- In Part B patients with AATD-associated liver disease, single doses of 30 mg and 60 mg BEAM-302 demonstrated consistent efficacy comparable to results observed in Part A patients without liver disease.

“These updated results, now with a robust clinical dataset from 29 patients, reinforce the potential for BEAM-302 to become a first- and best-in-class one-time treatment for patients with AATD,” said John Evans, chief executive officer of Beam Therapeutics. “Following a single dose, we observed significant increases in total AAT, production of corrected M-AAT and reductions in toxic Z-AAT, demonstrating successful correction of the underlying PiZ mutation and achievement of AAT levels consistent with MZ carriers who generally do not have either lung or liver disease in the absence of additional risk factors. The strength and consistency of this dataset support our selection of 60 mg as the go-forward dose and give us confidence in our ability to rapidly execute this next phase of pivotal development in pursuit of an accelerated approval pathway. We remain deeply committed to advancing this potentially transformative, one-time treatment for the AATD community.”

BEAM-302 Pivotal Development

Based on feedback from the U.S. Food and Drug Administration (FDA), Beam intends to pursue an accelerated approval pathway for BEAM-302 based on a primary endpoint of AAT biomarkers evaluated over 12 months, with 60 mg as the selected dose. To support a future biologics licensing application (BLA) submission, the company anticipates enrolling approximately 50 additional patients with AATD-associated lung disease, with or without liver disease, in an expansion of the ongoing open-label Phase 1/2 trial. Beam expects to initiate the pivotal cohort in the second half of 2026, leveraging its existing global clinical trial network.

Investor Webcast Information
Beam will host a conference call and webcast today, March 25, 2026, at 8:00 a.m. ET to review these updates. 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-302
BEAM-302 is a liver-targeting lipid-nanoparticle (LNP) formulation of base editing reagents designed to correct the PiZ mutation. Patients homozygous for this mutation (PiZZ) represent the majority of patients living with severe AATD disease. A one-time A-to-G correction of the PiZ mutation with Beam’s adenine base editor has the potential to simultaneously reduce the aggregation of mutant, misfolded AAT protein that causes toxicity to the liver (Z-AAT), generate therapeutic levels of corrected protein (M-AAT), and increase total and functional AAT in circulation, thereby addressing the underlying pathophysiology of both the liver and lung disease. In addition, the reduction in circulating PiZ has the potential to further minimize lung inflammation and dysfunction. Importantly, because BEAM-302 corrects the native AAT gene in its normal genetic location, AAT levels have been observed to increase physiologically in response to infection and inflammation in treated patients. This is a critical aspect of AAT’s normal function to regulate the body’s inflammatory response, which does not occur with currently approved protein replacement therapies. Correction of the PiZ mutation has been durable in patients treated in Beam's clinical trial.

About Alpha-1 Antitrypsin Deficiency (AATD)
AATD is an inherited genetic disorder that can cause early onset emphysema and liver disease. The most severe and common form of AATD arises when a patient has a point mutation in both copies of the *SERPINA1* gene at amino acid 342 position (E342K, also known as the PiZ mutation or the “Z” allele). This point mutation causes alpha-1 antitrypsin, or AAT, to misfold, accumulating inside liver cells rather than being secreted, resulting in very low levels (10%-15%) of circulating AAT. In addition to resulting in lower levels, the PiZ AAT protein variant is also less enzymatically effective compared to wildtype AAT protein (also known as the “M” allele). As a consequence, the lung is left unprotected from neutrophil elastase, resulting in progressive, destructive changes in the lung, such as emphysema, which can result in the need for lung transplant. The mutant AAT protein also accumulates in the liver, causing liver inflammation and cirrhosis, which can ultimately cause liver failure or cancer requiring patients to undergo a liver transplant. It is estimated that more than 100,000 individuals in the U.S. have two copies of the Z allele, known as the PiZZ genotype, although only about 10% of all patients are thought to have been diagnosed. Although augmentation therapy has been approved in the U.S. for the treatment of AATD-associated lung disease, there are currently no curative treatments and significant unmet need exists for patients with AATD.

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 AATD; our plans, and anticipated timing, to advance our AATD program; the clinical trial designs and expectations for BEAM-302; our anticipated regulatory interactions and filings; 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 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.

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[1] One patient dosed at 60 mg in Part B was not efficacy evaluable at the time of data cutoff.

[2] Steady state is defined as the period beginning on a patient's Day 28 visit and lasting until that patient's Month 12 visit (or until that patient's last visit, if earlier than twelve months).

[3] Circulating AAT levels measured using liquid chromatography–mass spectrometry (LC–MS) assay. LC-MS is a preferred quantitative method by regulatory authorities to assess specificity of AAT detection.