Beam Therapeutics Presents Updated Clinical Data from the Phase 1/2 Trial of BEAM-302 in Alpha-1 Antitrypsin Deficiency (AATD) at the European Respiratory Society (ERS) Congress 2026

September 8, 2026

BEAM-302 Continues to Demonstrate Potential as One-time Treatment for Both Lung and Liver Manifestations of AATD, with AAT Protein Production Under Normal Physiological Control

With Up to 18 Months Follow-Up, Data Show Long-Term Durability of In Vivo *Correction of Disease-causing Mutation with Single Dose of BEAM-302*

Treatment with BEAM-302 Increased Total and Functional AAT and Decreased Neutrophil Elastase Activity in Circulation

BEAM-302 Led to Reduction in Circulating Mutant Z-AAT and Corresponding Decrease in Toxic Circulating Z-polymers

Safety Profile of BEAM-302 Remains Consistent with LNP-based Therapies

Dosing Underway in Global Pivotal Cohort Designed to Support Potential U.S. Accelerated Approval

Beam to Host Investor Webcast Today, September 8, 2026, at 7:00 a.m. ET

CAMBRIDGE, Mass., Sept. 08, 2026 (GLOBE NEWSWIRE) -- Beam Therapeutics Inc. (Nasdaq: BEAM), a biotechnology company developing precision genetic medicines through base editing, today announced updated clinical data from the Phase 1/2 trial evaluating BEAM-302 for alpha-1 antitrypsin deficiency (AATD). The data were presented in a late-breaking oral presentation at the European Respiratory Society (ERS) Congress 2026 in Barcelona, Spain. BEAM-302 is Beam's lead liver-targeted genetic disease program and a potentially best- and first-in-class therapy designed to directly correct the root cause of AATD and address both liver and lung manifestations of disease.

"BEAM-302 is leading the way as a novel medicine that can potentially provide a genetic cure and treat both the lung and liver disease manifestations in patients living with severe AATD," said John Hurst, M.D., Ph.D., professor at University College London and an investigator in the BEAM-302 trial. "The data presented today at ERS are promising and demonstrate that a single dose of BEAM-302 restored AAT function, as evidenced by an increase in total circulating AAT above the protective threshold, the new production of corrected, functional M-AAT, significant reduction in mutant Z-AAT, and, importantly, AAT remained under normal physiologic control during periods of inflammation when it is needed."

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 is designed to evaluate patients with AATD-associated lung disease, and Part B evaluates patients with mild to severe liver disease, with or without lung disease. As of August 17, 2026, 38 patients have been dosed with BEAM-302 across Part A and Part B. The presentation at ERS included data from 29 patients treated with a single dose of BEAM-302 in the dose-escalation portion of the study: Part A (15 mg, n=3; 30 mg, n=3; 60 mg, n=6; 75 mg, n=9) and Part B (30 mg, n=3; 60 mg, n=5). Data from patients in the multi-dose cohort (n=3) and the Part A expansion cohort (n=6) were not included.

As of the June 24, 2026, data cutoff date, treatment with single-dose BEAM-302 was well tolerated with an acceptable safety profile in both Part A and Part B cohorts. Mild-to-moderate infusion-related reactions (IRRs) were the most common drug-related treatment-emergent adverse events (TEAEs), occurring in 41% of all patients. Transient and predominantly Grade 1 alanine transaminase (ALT) and/or aspartate aminotransferase (AST) elevations were also observed. One Part B patient with underlying AATD-related liver disease treated with 60 mg BEAM-302 experienced transient Grade 3 ALT/AST elevations that were not accompanied by bilirubin increases and did not require treatment.

Treatment with BEAM-302 led to rapid and durable increases of total and functional AAT, decreases in mutant Z-AAT and toxic Z-polymers, and new production of corrected M-AAT. Data were consistent with topline results announced in March 2026. Key findings from 29 efficacy-evaluable patients as of the June 24, 2026, data cutoff date include:

- A single dose of BEAM-302 of 60 mg led to sustained increases in total AAT above the 11 µM protective threshold. After treatment with a single dose of 60 mg BEAM-302 in Part A, the steady-state[1] circulating total AAT[2] mean and median were 14.4 µM and 15.2 µM, respectively, compared to 5.0 µM at baseline. In the Part B 60 mg cohort, the post-treatment steady-state circulating total AAT mean and median were 13.5 µM and 13.8 µM, respectively, compared to 4.7 µM at baseline.
- Increased total AAT in circulation was functional. Functional AAT levels increased in a dose-dependent manner up to 60 mg and were durable through follow-up.
- Human neutrophil elastase (HNE) activity levels, another measure of AAT function, decreased in a dose proportionate manner with BEAM-302 treatment, with >80% of patients in the Part A 60 mg cohort having at least one HNE activity measurement below the lower limit of quantification.
- Mutant Z-AAT was significantly and durably reduced after treatment with BEAM-302, with an 84% reduction in circulating Z-AAT in both Part A and Part B 60 mg cohorts.
- BEAM-302 also led to a significant decrease in circulating Z-AAT protein aggregates, referred to as Z-polymers, from

baseline in the 60 mg cohort. Circulating Z-polymers have been shown to correlate with liver disease severity[3] and to amplify lung inflammation[4] by inducing neutrophil recruitment.

- Evidence of AAT protein production under normal physiologic control was observed during an upper respiratory tract infection in a patient in the Part A 60 mg cohort.
- Following treatment with BEAM-302, newly produced corrected M-AAT comprised the majority of AAT in circulation. The proportion of M-AAT at steady state was 93% following treatment with 60 mg in both Part A and Part B, exceeding the approximately 80% M-AAT proportion associated with the MZ genotype.

“These data reinforce the potential of BEAM-302 to address AATD at its root cause by correcting the disease-causing mutation and restoring production of functional AAT needed to prevent lung and liver damage with a single treatment,” said Amy Simon, M.D., chief medical officer of Beam Therapeutics. “With our global pivotal cohort now dosing patients, we are building on our scientific and clinical leadership in the field as we advance the only genetic medicine in pivotal development for AATD. We believe BEAM-302 has the potential to transform the AATD treatment paradigm, and we are focused on bringing this one-time treatment to patients who have been waiting decades for more treatment options that can impact the spectrum of disease manifestations.”

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 license 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. In July, Beam dosed the first patient in this global pivotal cohort.

Investor Webcast Information

Beam will host a conference call and webcast today, September 8, 2026, at 7:00 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 to date 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 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 pursuit of an accelerated approval pathway for BEAM-302 based on biomarker endpoints; our competitive position in the AATD treatment landscape; 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; that the FDA may not agree to an accelerated approval pathway for BEAM-302 or may require additional data or studies; 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, 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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[1] Steady state is defined as the period beginning on a patient's Day 28 visit and lasting until that patient's last visit.

[2] Circulating total AAT levels were measured using turbidimetry.

[3] Mahadeva, Ravi et al. “Polymers of Z alpha1-antitrypsin co-localize with neutrophils in emphysematous alveoli and are chemotactic in vivo.” *The American journal of pathology* vol. 166,2 (2005): 377-86.

[4] Mulgrew, Alan T et al. “Z alpha1-antitrypsin polymerizes in the lung and acts as a neutrophil chemoattractant.” *Chest* vol. 125,5 (2004): 1952-7.