



Beam Therapeutics to Present Updated Data from Phase 1/2 Trial of BEAM-302 in Alpha-1 Antitrypsin Deficiency (AATD) in Late-Breaking Oral Presentation at the European Respiratory Society (ERS) Congress 2026

July 23, 2026

Beam to Host Investor Webcast to Review BEAM-302 Clinical Data on Tuesday, September 8, 2026, at 7:00 a.m. ET

CAMBRIDGE, Mass., July 23, 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 updated clinical data from the Phase 1/2 trial evaluating BEAM-302 for alpha-1 antitrypsin deficiency (AATD) at the European Respiratory Society (ERS) Congress 2026, taking place September 5–9, 2026, in Barcelona, Spain. BEAM-302 is Beam's lead genetic disease program designed to be a best-in-class and first-in-class liver-targeting therapy for AATD that addresses the underlying pathophysiology of both liver and lung disease.

"There remains a significant need for new treatment options for people living with AATD, as no therapies are available today that simultaneously restore AAT levels and function while eliminating toxic Z-AAT, thereby addressing both the lung destruction and liver damage that occurs," said Amy Simon, M.D., chief medical officer of Beam. "BEAM-302 is the most advanced genetic medicine in clinical development for AATD, and we are excited to share a robust updated dataset at the ERS Congress that reinforces its potential to transform outcomes for patients. As we prepare to initiate the pivotal cohort, we remain focused on bringing this one-time treatment to patients as quickly and as safely as possible."

Details are as follows:

Title: First-in-Human in vivo Gene Editing for Severe Alpha-1 Antitrypsin Deficiency (AATD): Initial Data from the Phase 1/2 Study of BEAM-302

Presentation Session: Understanding Treatment Response in Airway Diseases: from Mechanisms to Clinical Biomarkers

Presentation Session Time: Tuesday, September 8, 2026, 9:30–10:45 a.m. CEST

Presenter: John Hurst, M.D., Ph.D., University College London

Investor Webcast Information

Beam will host a conference call and webcast on Tuesday, 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 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 expected presentation at the ERS conference; 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, our Quarterly Report on Form 10-Q for the quarter ended March 31, 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