Beam Therapeutics Presents Recently Reported Topline Clinical Data for BEAM-302 in Alpha-1 Antitrypsin Deficiency (AATD) at the American Thoracic Society (ATS) 2026 International Conference

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Presentation Features Additional Data from the Single-dose Cohorts of the Phase 1/2 Trial, Including Detailed Safety Results, Efficacy Durability and Reduction in Human Neutrophil Elastase Activity Post-BEAM-302 Treatment

CAMBRIDGE, Mass., May 18, 2026 (GLOBE NEWSWIRE) -- Beam Therapeutics Inc. (Nasdaq: BEAM), a biotechnology company developing precision genetic medicines through base editing, today presented the recently reported clinical data from the BEAM-302 Phase 1/2 trial in alpha-1 antitrypsin deficiency (AATD) at a symposium on translating scientific discovery in gene editing into clinical progress for patients with lung disease. The presentation was given by Amy Simon, M.D., chief medical officer of Beam, at the American Thoracic Society (ATS) International Conference being held in Orlando, Fla.

“At Beam, we are committed to leading innovation in the AATD community, with a goal of transforming disease outcomes for all patients suffering from this disease,” said Dr. Simon. “For BEAM-302, the data shared today build on the growing body of clinical evidence that supports the profound impact of treating AATD at the root cause of disease, the DNA mutation, with this one-time investigational therapy. We are rapidly executing toward pivotal development to deliver BEAM-302 to patients with AATD as safely and expeditiously as possible. Our long-term goal is to combine our growing understanding of AATD biology and our leading gene editing capabilities to maximize patient benefit across the entire spectrum of disease manifestations. We are also continuing to expand our cross-sector collaborations with leading AATD advocacy organizations to advance disease awareness and increase diagnosis, support the evolution of research approaches and incorporate patient perspectives across the broader scientific and care community.”

“The ongoing results from the BEAM-302 trial are truly remarkable, suggesting a single treatment dose can correct AATD at its root cause and durably restore normal AAT function, addressing both lung and liver manifestations of disease over a patient's entire lifetime,” said John Hurst, M.D., Ph.D., professor at the University College London and an investigator in the BEAM-302 trial. “This is not only a paradigm shift for the treatment of AATD, but also for medicine more widely as we enter the era of gene correction as a tool for clinicians.”

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. Topline data from 29 patients treated with BEAM-302 as of a February 10, 2026 data cutoff date were reported in March 2026. Dr. Simon's presentation at ATS features additional data for the single-dose cohorts from the same data cutoff, including detailed safety results, efficacy durability and reduction in human neutrophil elastase activity (a direct measure of AAT function) post-BEAM-302 treatment. Dr. Simon’s presentation is available on the “Presentations and Publications” section of Beam’s website at beamtx.com.

Based on feedback from the U.S. Food and Drug Administration (FDA), Beam intends to pursue an accelerated approval pathway for BEAM-302. 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 this pivotal cohort in the second half of 2026. In addition, Beam expects to present detailed and updated BEAM-302 data at a medical congress in 2026.

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, 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.

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