Beam Therapeutics Sets Strategic Priorities for its Genetic Disease and Hematology Franchises to Drive Execution of Late-Stage Clinical Programs and Extends its Operating Runway through Commercial Transition

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Alignment Reached with U.S. FDA on Potential Accelerated Approval Pathway for BEAM-302 in Alpha-1 Antitrypsin Deficiency (AATD) Based on Biomarker Endpoints

U.S. Biologics Licensing Application (BLA) Submission for risto-cel (Previously Known as BEAM-101) Expected as Early as Year-End 2026

Expansion of Liver-targeted Genetic Disease Franchise Underway with New Program to be Announced in First Half of 2026

Ended 2025 with Estimated $1.25 Billion in Cash, Cash Equivalents and Marketable Securities; Projected Operating Runway Extension into 2029 Supports Anticipated risto-cel Launch and Execution of BEAM-302 Pivotal Development Plan

CAMBRIDGE, Mass., Jan. 11, 2026 (GLOBE NEWSWIRE) -- Beam Therapeutics Inc. (Nasdaq: BEAM) today announced continued progress toward its mission to build a sustainable, predictable model for the advancement of precision genetic medicines, highlighting recent updates for its liver-targeted genetic disease and hematology franchises and strategic priorities in 2026, supported by an extended anticipated operating runway.

“Over the past year, we have continued to demonstrate the power and consistency of our base editing platform as we work to redefine what is possible in genetic medicine,” said John Evans, chief executive officer of Beam Therapeutics. “Our approach is rooted in precision and predictability – designing one-time treatments to reverse genetic disease, executing against our portfolio priorities with discipline, and generating differentiated clinical data that compound across programs. As our science and company matures, each milestone strengthens the foundation for the next, enabling us to advance a growing pipeline of programs with increasing confidence and speed. We are now further extending this platform-driven rigor into regulatory execution, including alignment with the FDA on a potential path to accelerated approval for BEAM-302 and a planned BLA submission for risto-cel as early as the end of 2026. Backed by a strong balance sheet and expected cash runway into 2029, we believe we are uniquely positioned to translate scientific innovation into meaningful, lasting benefit for patients with serious diseases.”

Recent Pipeline Updates and 2026 Anticipated Milestones

Liver-targeted Genetic Disease Franchise

Beam is building a platform approach for single-course, precision gene editing therapies for liver-targeted genetic diseases by delivering base editors through intravenous infusion of lipid-nanoparticles (LNPs), a clinically validated technology for delivery of nucleic acid payloads to the liver.

BEAM-302: Beam’s lead genetic disease program is designed to be a best-in-class and first-in-class liver-targeting therapy for alpha-1 antitrypsin deficiency (AATD) that addresses the underlying pathophysiology of both liver and lung disease. In an open-label Phase 1/2 clinical trial, treatment with BEAM-302 demonstrated the first-ever clinical *in vivo* genetic correction of a disease-causing mutation and established clinical proof of concept in AATD.

- To date, more than 25 AATD patients with lung and/or liver disease have been treated in the dose-exploration portions of the trial.
- Beam has reached alignment with the U.S. Food and Drug Administration (FDA) on a potential accelerated approval pathway for BEAM-302 based on AAT biomarkers evaluated over 12 months. To support a future BLA submission, the company anticipates enrolling approximately 50 additional patients to be treated with the selected optimal biologic dose of BEAM-302 in an expansion of the ongoing Phase 1/2 study.
- BEAM-302 has also been accepted into the FDA’s Chemistry, Manufacturing, and Controls Development and Readiness Pilot (CDRP) program aimed at facilitating CMC development of products with expedited clinical development timeframes.
- Beam expects to report updated data from the Phase 1/2 trial and next steps for pivotal development by the end of the first quarter of 2026.

BEAM-301: BEAM-301 aims to correct the most common disease-causing mutation, R83C, in patients with glycogen storage disease type Ia (GSDIa). BEAM-301 has the potential to normalize blood glucose in these patients without continuous supplementation and improve key metabolic parameters.

- BEAM-301 is currently being evaluated in an open-label Phase 1/2 dose-exploration trial in patients with GSDIa. Dosing is complete in the first cohort and enrollment has been initiated in the second cohort.
- Beam expects to report initial clinical data in 2026.

Pipeline Expansion: Beam expects to disclose the next clinical program for its liver-targeted genetic disease franchise in the first half of 2026.

Hematology Franchise

Beam is pursuing a long-term, multi-wave development strategy for sickle cell disease (SCD), intended to progressively expand the reach of the company's base editing approach to broader subsets of patients.

Risto-cel: Ristoglogene autogetemcel (risto-cel, formerly known as BEAM-101) is an investigational autologous cell therapy with a potential best-in-class profile for the treatment of SCD. The most recent data from the ongoing BEACON Phase 1/2 clinical trial presented at the 67th American Society of Hematology (ASH) Annual Meeting and Exposition continue to show evidence of risto-cel's differentiated treatment profile. Risto-cel has demonstrated a deeper resolution of SCD markers and reduced time in the hospital driven by a median of one cell collection cycle, rapid engraftment, and low number of neutropenic days. Importantly, risto-cel's predictable, robust manufacturing process led to fewer mobilizations and rapid delivery from cell collection to dose, potentially improving patient experience as well as treatment center capacity.

- The adult and adolescent cohorts of the BEACON trial were fully enrolled in mid-2025.
- Manufacturing of all doses was completed as of December 2025.
- In addition, Beam completed interactions with the FDA on the anticipated BLA package for risto-cel, which is expected to align with regulatory precedent set by previously approved SCD gene therapies.
- Risto-cel has also been accepted into the FDA's CDRP program.
- Beam expects to submit a BLA for risto-cel as early as year-end 2026.

Next-generation Programs in Sickle Cell Disease and Hematology: Beam continues to make significant investments in developing targeted LNPs for delivery of genetic payloads outside of the liver. Based on recent advances using this technique to deliver gene editing to hematopoietic stem cells (HSCs), Beam is now prioritizing *in vivo* delivery for its next wave approach in SCD, complementing risto-cel as a potential best-in-class *ex vivo* therapy. Beam's long-term goal is to provide accessible, transformative genetic therapies for all patients with SCD. Beam is also continuing development of its proprietary ESCAPE platform, a powerful technology to potentially enable non-genotoxic treatment strategies that can be delivered either *ex vivo* or *in vivo*, including as part of any future *in vivo* program for SCD.

- The ongoing Phase 1 healthy volunteer clinical trial of BEAM-103, an anti-CD117 monoclonal antibody (mAb) that enables ESCAPE, is expected to complete dosing in the first half of 2026.
- Multiple targeted LNPs for HSC delivery have been identified and are in lead optimization.

Cash Position and Updated Operating Runway
Beam estimates that it had $1.25 billion in cash, cash equivalents and marketable securities as of December 31, 2025. This estimate is inclusive of the $255.1 million in closing cash consideration received from the acquisition of Orbital Therapeutics by Bristol-Myers Squibb. In addition, Beam has the right to receive up to approximately $26.3 million in additional cash consideration upon the release, if any, of certain escrows from the transaction. This estimate of cash on hand is preliminary, unaudited and subject to completion of Beam's financial statement closing procedures. This estimate does not present all information necessary for an understanding of Beam's financial condition as of December 31, 2025, and its results of operations for the three months and year ended December 31, 2025. Accordingly, undue reliance should not be placed on this preliminary estimate.

Beam now expects that its estimated cash, cash equivalents and marketable securities as of December 31, 2025, will enable the company to cover its anticipated operating expenses and capital expenditure requirements into 2029, funding the company through the anticipated launch of risto-cel in SCD and execution of the BEAM-302 pivotal development plan in AATD.

J.P. Morgan Healthcare Conference
Management will present and discuss Beam's pipeline and business updates during a presentation at the 44th Annual J.P. Morgan Healthcare Conference on Tuesday, January 13, 2026, at 5:15 p.m. PT. A live webcast will be available in the investor section of the company's website at www.beamtx.com and will be archived for 60 days following the presentation.

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 life-long 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: our upcoming presentations at the 44th Annual J.P. Morgan Healthcare Conference; the therapeutic applications and potential of our technology, including with respect to SCD, AATD and GSDIa; our plans, and anticipated timing, to advance our programs and present data from ongoing clinical trials; the clinical trial designs and expectations for risto-cel, BEAM-103, BEAM-301 and BEAM-302; our anticipated regulatory interactions and filings; our estimated cash, cash equivalents and marketable securities as of December 31, 2025 and our expectations related thereto; our potential receipt of additional cash consideration upon the release, if any, of certain escrows relating to the acquisition of Orbital Therapeutics by Bristol-Myers Squibb; the sufficiency of our capital resources to fund operating expenses and capital expenditure requirements and the period in which such resources are expected to be available; and our ability to develop life-long, 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; whether our actual audited results will be consistent with our estimated cash, cash equivalents and marketable securities as of December 31, 2025; 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, 2024, our Quarterly Report on Form 10-Q for the quarter ended September 30, 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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