Beam Therapeutics Announces Progress in Hematology and Genetic Disease Franchises and Outlines Key 2025 Anticipated Catalysts

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More Than 40 Adult Sickle Cell Disease Patients Now Enrolled in BEACON Trial of BEAM-101; Beam Expects to Dose 30 Patients and Present Updated Data by Mid-2025

Initial Data from Phase 1/2 Trial of BEAM-302 in Alpha-1 Antitrypsin Deficiency Expected in First Half 2025

Dosing Anticipated to Commence in Phase 1/2 Trial of BEAM-301 in Glycogen Storage Disease Type 1a in Early 2025

IND-enabling Studies of ESCAPE Nongenotoxic Conditioning Approach Underway, with Healthy Volunteer Study of BEAM-103 Antibody Expected to Initiate by Year-end

Cash Runway Expected to Support Operating Plans into 2027, Now Inclusive of Commercial Readiness Activities for BEAM-101

Cambridge, Mass., Jan. 13, 2025 (GLOBE NEWSWIRE) -- Beam Therapeutics Inc. (Nasdaq: BEAM), a biotechnology company developing precision genetic medicines through base editing, today announced progress across the company's hematology and genetic disease franchises and provided updates on anticipated upcoming milestones.

"We're entering 2025 at an important inflection point in the evolution of Beam, having advanced four programs into the clinic, established clinical differentiation for base editing with our lead sickle cell disease program, and prioritized two high-value core franchises with best-in-class potential – all of the key attributes needed to create a long-term leading company in gene editing," said John Evans, chief executive officer of Beam. "Importantly, we remain in a strong financial position, with our core manufacturing, regulatory and clinical capabilities now in place. This year, we are poised to deliver critical data and achieve key milestones across our pipeline, which we expect will bring us closer to our mission of offering life-long cures for patients in need."

Pipeline Updates and 2025 Anticipated Milestones

Hematology Franchise

Beam is pursuing a long-term, staged development strategy for sickle cell disease (SCD) that includes three "waves" of innovation intended to progressively expand the reach of the company's base editing approach to broader subsets of patients.

BEAM-101: Wave 1 gene editing treatments aim to deliver a genetically modified cell product through stem cell transplant, enabled by chemotherapy conditioning, for the most severe SCD patients. Beam's wave 1 approach is BEAM-101, an autologous investigational cell therapy designed to efficiently and uniformly increase fetal hemoglobin (HbF) in red blood cells without relying on double-stranded breaks, offering a potentially best-in-class profile. BEAM-101 is being evaluated in the BEACON Phase 1/2 clinical trial, and initial results were presented at the 66th American Society of Hematology (ASH) Annual Meeting and Exposition in December 2024.

- To date, more than 40 adult patients with SCD have cleared screening and enrolled in BEACON, and, of these, 13 have been dosed. Beam expects to dose 30 patients by mid-2025.
- The U.S. Food and Drug Administration (FDA) and the BEACON data monitoring committee approved enrollment of adolescent SCD patients ages 12-17 years in the study, and screening has commenced.
- Beam expects to present updated data from the BEACON trial in mid-2025.

ESCAPE: Beam's wave 2 approach is its Engineered Stem Cell Antibody Evasion (ESCAPE) platform, which aims to provide the same *ex vivo*-manufactured cell product deployed in wave 1, but now using a non-genotoxic alternative to traditional transplant myeloablative conditioning. Proof-of-concept data in non-human primates (NHPs) demonstrating engraftment of base-edited cells using antibody conditioning were presented at ASH. Beam plans to develop the ESCAPE technology initially in SCD and beta-thalassemia as well as potential future hematology indications.

- In December, Beam initiated Phase 1-enabling preclinical toxicology studies for ESCAPE.
- The company expects to initiate a Phase 1 healthy volunteer clinical trial of BEAM-103, an anti-CD117 monoclonal antibody (mAb) designed to suppress hematopoietic stem and progenitor cells that express CD117, by the end of 2025.

In vivo: In wave 3, Beam is exploring the potential for *in vivo* base editing programs for SCD, in which base editors would be delivered to the patient through intravenous infusion of lipid nanoparticles (LNPs) targeted to hematopoietic stem cells, eliminating the need for transplantation altogether.

Genetic Disease Franchise

Beam's second core area of focus seeks to create single-course gene editing therapies for genetic diseases by delivering base editors through intravenous infusion of LNPs, which are a clinically validated technology for delivery of nucleic acid payloads to the liver.

BEAM-302: Beam's lead genetic disease program is BEAM-302, a potentially best-in-class liver-targeting LNP formulation of base editing reagents designed to correct the PiZ allele, the most common gene variant associated with severe alpha-1 antitrypsin deficiency (AATD). BEAM-302 has the

potential to simultaneously reduce the aggregation of mutant, misfolded AAT protein that causes toxicity to the liver and increase circulating levels of corrected and functional AAT protein, thus addressing the underlying pathophysiology of both the liver and lung disease. BEAM-302 is being evaluated in a Phase 1/2 dose-escalation clinical trial.

- The company continues to advance global regulatory and site activation activities with sites now open in the United Kingdom, New Zealand, Australia and Netherlands.
- Beam expects to report initial data from multiple cohorts from the Phase 1/2 study in the first half of 2025.

BEAM-301: BEAM-301 is a liver-targeting LNP formulation of base editing reagents designed to correct the R83C mutation, the most common disease-causing mutation that results in the most severe form of glycogen storage disease type 1a (GSD1a). GSD1a is an autosomal recessive disorder caused by mutations involved in maintaining glucose homeostasis and is associated with life-threatening fasting hypoglycemia as well as long-term complications impacting the liver and kidney. BEAM-301 has the potential to normalize blood glucose without continuous supplementation and improve metabolic parameters. BEAM-301 is being evaluated in a Phase 1/2 dose-escalation clinical trial.

- The first clinical trial site for the Phase 1/2 clinical trial of BEAM-301 is now active, with patient dosing expected to commence in early 2025.

Partnered Programs: Beam continues to progress its research collaborations with Pfizer and Apellis. Under the Apellis collaboration, which is focused on multiple base editing programs that target specific genes within the complement system, the companies are advancing preclinical studies for a one-time treatment targeting the neonatal Fc receptor (FcRn) using gene editing technology from Beam.

Cash Position and Updated Operating Runway
As of December 31, 2024, Beam estimates that it had $850.7 million in cash, cash equivalents and marketable securities. This estimate is preliminary, unaudited and is subject to completion of Beam's financial statement closing procedures. This estimate also does not present all information necessary for an understanding of Beam's financial condition as of December 31, 2024, and its results of operations for the three months and year ended December 31, 2024. 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, 2024, will enable the company to fund its anticipated operating expenses and capital expenditure requirements into 2027, inclusive of commercial spend related to the potential launch of BEAM-101.

J.P. Morgan Healthcare Conference
Beam management will present and discuss Beam's pipeline and business updates during a presentation at the 43rd Annual J.P. Morgan Healthcare Conference today, Monday, January 13, 2025, at 1:30 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 43rd Annual J.P. Morgan Healthcare Conference; the therapeutic applications and potential of our technology, including with respect to SCD, AATD, GSD1a and beta thalassemia; our plans, and anticipated timing, to advance our programs; the clinical trial designs and expectations for BEAM-101, BEAM-103, BEAM-301 and BEAM-302; our estimated cash, cash equivalents and marketable securities as of December 31, 2024 and our expectations related thereto; 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, 2024; 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, 2023, our Quarterly Report on Form 10-Q for the quarter ended September 30, 2024, 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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