Beam Therapeutics Reports Progress Across Base Editing Portfolio and Outlines Key Anticipated Milestones

January 9, 2023

BEACON Trial of BEAM-101 in Sickle Cell Disease Ongoing with Data from Multiple Patients Expected in 2024; Expansion Phase Initiation Expected in 2023

First Patient Dosing in BEAM-201 Trial in Patients with T-ALL/T-LL Expected by Mid-2023

Regulatory Submissions Planned for BEAM-301 by Late 2023/Early 2024 and BEAM-302 in Early 2024

North Carolina Manufacturing Facility Expected to Initiate GMP Operations in Late 2023

Approximately $1 Billion Estimated Cash, Cash Equivalents and Marketable Securities at Year-End 2022; Cash Runway Expected into 2025, through Anticipated Key Milestones for Lead Programs and Long-Term Platform Opportunities

CAMBRIDGE, Mass., Jan. 09, 2023 (GLOBE NEWSWIRE) -- Beam Therapeutics Inc. (Nasdaq: BEAM), a biotechnology company developing precision genetic medicines through base editing, today reported progress across the company's hematology, immunology-oncology and genetic disease portfolios and provided updates on anticipated upcoming milestones.

"Beam enters 2023 with significant momentum across all of our core pipeline areas and an expanding leadership position in the next generation of gene editing," said John Evans, chief executive officer of Beam. "We have multiple clinical-stage candidates with BEAM-101 and BEAM-201, another two candidates – BEAM-301 and BEAM-302 – moving toward clinical trials, and an integrated platform of editing technologies, scalable manufacturing capabilities and diverse delivery modalities. We are also making key investments in long-term platform opportunities that may dramatically expand the reach and impact of base editing and create a sustainable pipeline of highly differentiated programs, including improved conditioning for transplant, allogeneic cell therapies and *in vivo* delivery. I'm so proud of the accomplishments this organization has achieved in our first five years of operations. We believe we now have a unique opportunity to deliver on the potential of our science and achieve our mission of bringing life-changing treatments to patients suffering from serious diseases."

Hematology Portfolio

- **BEAM-101:** In November 2022, Beam enrolled the first patient in its BEACON clinical trial evaluating BEAM-101 as a treatment for sickle cell disease (SCD). Beam expects to complete enrollment in the sentinel cohort and initiate enrollment in the expansion cohort of BEACON in 2023, with plans to report data from multiple patients from one or both cohorts in 2024. BEAM-101 is a patient-specific, autologous hematopoietic stem cell (HSC) investigational therapy designed to offer a potentially best-in-class profile, incorporating base edits that are intended to mimic single nucleotide polymorphisms seen in individuals with hereditary persistence of fetal hemoglobin. BEAM-101 aims to potentially alleviate the effects of SCD or beta-thalassemia by leading to increases in fetal hemoglobin, which is expected to restore the formation of a functional hemoglobin tetramer and, in the case of SCD, inhibit hemoglobin S polymerization.
- **Platform Opportunity:** Beam is advancing its Engineered Stem Cell Antibody Paired Evasion (ESCAPE) conditioning strategy in an effort to bring base editing treatments to more patients. ESCAPE aims to avoid toxicity challenges associated with currently available conditioning regimens for patients with SCD and beta-thalassemia ahead of autologous transplant. ESCAPE may also have applications in other diseases of the blood and immune system where transplant could deliver potential benefits but has been limited by toxicities associated with standard conditioning regimens. In December 2022, Beam presented *in vivo* proof-of-concept data at the American Society of Hematology Annual Meeting and Exposition (ASH) highlighting its potential. Beam has made significant investments in its ESCAPE platform and plans to continue its advancement in 2023.

Immunology-oncology Portfolio

- **BEAM-201:** In December 2022, Beam received clearance from the FDA for its Investigational New Drug (IND) application for BEAM-201. The company has initiated a first-in-human Phase 1/2 clinical trial to evaluate the safety and efficacy of BEAM-201 in patients with relapsed/refractory T-cell acute lymphoblastic leukemia (T-ALL)/T-cell lymphoblastic lymphoma (T-LL) and expects to dose the first patient by mid-2023. The Phase 1 portion of the trial is expected to include up to 48 patients between the ages of 18 and 50, followed by a Phase 2 portion with approximately 48 patients. Key safety endpoints for the trial include treatment-emergent and treatment-related adverse events, and key efficacy endpoints include proportion of patients with complete or partial responses, proportion eligible for HSC transplant, and proportion achieving minimal residual disease negative status. Beam believes that BEAM-201 is the first quadruple-edited, allogeneic CAR-T cell investigational therapy in clinical-stage development. BEAM-201 is designed to target CD7 to treat relapsed/refractory

T-ALL/T-LL, a severe disease affecting children and adults.
- **Platform Opportunity:** Beyond BEAM-201, Beam continues to research potential next-generation allogeneic strategies that could dramatically expand the utility and accessibility of cell therapies in cancer and other diseases. Beam anticipates that multiple edits will be required to enable allogeneic cells to successfully avoid immune rejection and provide the cells with other desirable properties. Beam believes that multiplex base editing, with its high potency, efficiency in editing and lack of double-strand breaks, is well suited for making such highly engineered cells, and anticipates providing additional updates on this research in 2023.

Genetic Disease Portfolio

- **BEAM-301:** IND-enabling studies for BEAM-301 continue, and by late 2023 or early 2024, the company plans to submit a regulatory application for authorization to initiate clinical trials for the program. BEAM-301 is a liver-targeting lipid nanoparticle (LNP) formulation of base editing reagents designed to correct the R83C mutation, the most common disease-causing mutation which results in the most severe form of glycogen storage disease 1a (GSDIa). GSDIa is an autosomal recessive disorder caused by mutations in the G6PC gene that disrupt a key enzyme, glucose-6-phosphatase, critical for maintaining glucose homeostasis. Patients with this mutation typically require ongoing corn starch administration, without which, they may enter into hypoglycemic shock within one to three hours.
- **BEAM-302:** Beam also continues to advance its second liver-targeted *in vivo* program, BEAM-302, and in early 2024, plans to submit a regulatory application for authorization to initiate clinical trials for the program. BEAM-302 is designed to offer a one-time treatment to genetically correct the E342K point mutation (PiZZ genotype), which is most commonly responsible for severe alpha-1 antitrypsin deficiency (AATD). AATD is an inherited genetic disorder that can cause early onset emphysema and liver disease.
- **Platform Opportunity:** Beam continues to advance its LNP delivery technologies using its barcode screening technology, which is designed to enable delivery of base editing treatments to the liver and tissues beyond, potentially expanding the number of diseases and patients that could benefit from base editing medicines.

Manufacturing Facility

- Beam continues to expect operations at its North Carolina manufacturing facility to commence in the first quarter of 2023 and expects to initiate current good manufacturing practice compliant operations in late 2023.

Cash Position and Runway

- **Cash Position**: Beam estimates that it had cash, cash equivalents and marketable securities of approximately $1.0 billion as of December 31, 2022. 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, 2022, and its results of operations for the three months and year ended December 31, 2022. Accordingly, undue reliance should not be placed on this preliminary estimate.
- **Cash Runway**: Beam expects that its cash, cash equivalents and marketable securities as of December 31, 2022, will enable the company to fund its anticipated operating expenses and capital expenditure requirements at least into 2025. This expectation includes funding directed toward reaching each of the key milestones for BEAM-101, BEAM-201, BEAM-301 and BEAM-302 described above, as well as continued investments in platform advancements and manufacturing capabilities.

J.P. Morgan Healthcare Conference

Beam management will present and discuss Beam's pipeline and business updates during a presentation at the 41 st Annual J.P. Morgan Healthcare Conference today, Monday, January 9, 2023, at 11:15 a.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 that includes a suite of gene editing and delivery technologies and is in the process of building 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 41st Annual J.P. Morgan Healthcare Conference; our expectations for transitioning to a multi-program clinical stage company; the therapeutic applications and potential of our technology, including with respect to SCD, GSDIa, T-ALL/TLL, AATD and our conditioning regimens; the clinical trial design for BEAM-201; our plans, and anticipated timing, to advance our programs; our estimated cash, cash equivalents and marketable securities as of December 31, 2022 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 potential impact of the COVID-19 pandemic, including its impact on the global supply chain; the uncertainty that our product candidates will receive regulatory approval necessary to initiate human clinical studies; 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 enrollment and initiation of our clinical trials may take longer than expected; 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, 2022; 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, 2021, our Quarterly Report on Form 10-Q for the quarter ended September 30, 2022, 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.

This press release contains hyperlinks to information that is not deemed to be incorporated by reference in this press release.

Contacts:

Investors:
Chelcie Lister
THRUST Strategic Communications
chelcie@thrustsc.com

Media:
Dan Budwick
1AB
dan@1abmedia.com