Beam Therapeutics Highlights Progress Across Base Editing Portfolio and Outlines 2024 Anticipated Milestones

January 8, 2024

First Patient Dosed and Successfully Engrafted in BEACON Phase 1/2 Trial of BEAM-101 in Patients with Severe Sickle Cell Disease; Significant Enrollment Progress Supports First Expected Clinical Data Readout in Second Half of 2024

European Clinical Trial Application (CTA) Submitted for BEAM-302; Trial Initiation in Alpha-1 Antitrypsin Deficiency Planned for First Half of 2024

Investigational New Drug (IND) Application for BEAM-301 On-track for First Half of 2024

Cash Runway Expected to Support Operating Plans into 2027

CAMBRIDGE, Mass., Jan. 08, 2024 (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 and genetic disease portfolios and provided updates on anticipated upcoming milestones.

"Our vision is to establish Beam as a sustainable, fully integrated company pioneering a new class of genetic medicines with base editing. We made tremendous progress toward this goal in 2023, including opening our own GMP manufacturing facility, dosing the first patients in multiple *ex vivo* clinical programs, including BEAM-101 for sickle cell disease (SCD), and accelerating our *in vivo* program for alpha-1 antitrypsin deficiency (AATD), as exemplified by the filing of our CTA for BEAM-302," said John Evans, chief executive officer of Beam Therapeutics. "Building on this momentum and benefiting from the significant clinical validation, regulatory clarity, and scientific breakthroughs occurring in the broader gene editing field, we expect 2024 to be a year of significant catalysts for Beam. Our highly differentiated SCD and AATD programs have the potential to provide best-in-class therapies for significant patient populations with high unmet need, while also establishing a platform for sustainable long-term growth across multiple therapeutic areas."

Pipeline Updates and 2024 Anticipated Milestones

Sickle Cell Disease (SCD) Franchise

Beam is pursuing a long-term, staged development strategy for SCD that has three Waves of innovation intended to progressively expand the reach of our base editing approach to broader subsets of patients.

- **Wave 1:** BEAM-101 is 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. Preclinical models suggest base editing could lead to improved HbF induction and lower residual disease-causing hemoglobin S compared to existing gene therapy options.
    - The first patient was dosed in the fourth quarter of 2023 and successfully achieved engraftment in the BEACON Phase 1/2 clinical trial, an open-label, single-arm, multicenter study evaluating the safety and efficacy of BEAM-101 in adult patients with severe SCD. Treatment with BEAM-101, in which the edited cell product is delivered in an autologous bone marrow transplant, will occur on a sequential basis for the first three patients treated in the trial, and then will be given in parallel for all subsequent patients.
    - Patients have continued to be consented in the BEACON trial, and Beam anticipates dosing the remaining two patients in the sentinel cohort and initiating dosing in patients in the expansion cohort in the first half of 2024.
    - The company is on-track to report initial data on multiple patients from the BEACON trial in the second half of 2024.
- **Wave 2:** Beam continues to advance and invest in its Engineered Stem Cell Antibody Paired Evasion (ESCAPE) conditioning platform and anticipates initiating Phase 1-enabling preclinical studies for the program in 2024. ESCAPE aims to avoid the toxicities associated with currently available conditioning regimens for patients with SCD required prior to autologous transplant.
- **Wave 3:** The company is also 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 seeks to treat genetic diseases using single course gene editing therapies delivered through intravenous infusion of LNPs, which are a clinically validated technology for delivery of nucleic acid payloads to the liver.

- BEAM-302, the company's priority genetic disease program, is a potential treatment for AATD, which is characterized by early onset emphysema, liver disease, and increased all-cause mortality compared to the general population. There is a

large unmet need for novel therapies that can treat patients with AATD-associated lung and liver disease.
    - BEAM-302 is 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 AATD. Approximately 100,000 patients in the U.S. are estimated to carry the PiZZ genotype.
    - Preclinical data to date demonstrated that treatment with BEAM-302 led to significantly increased levels of corrected and functional alpha-1 antitrypsin (AAT) and reduced mutant PiZ AAT in multiple *in vivo* rodent disease models at clinically relevant doses. These findings support the potential of BEAM-302 to efficiently correct the disease-causal PiZ mutation after a single dose and potentially address both the liver and lung disease associated with AATD.
    - Beam has filed a CTA for BEAM-302, and, assuming CTA acceptance, plans to initiate a clinical trial for BEAM-302 in the first half of 2024.
- In addition, Beam is also advancing BEAM-301 for the potential treatment of glycogen storage disease type 1a (GSD1a), an autosomal recessive disorder caused by mutations in the G6PC gene that disrupt a key enzyme, glucose-6-phosphatase, involved in maintaining glucose homeostasis.
    - 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 GSD1a.
    - Preclinical data have shown that a single administration of BEAM-301 directly and durably corrected the R83C mutation *in vivo*, with an ongoing significant survival benefit one year after the initial dosing**.**
    - Beam is focusing initial development of BEAM-301 in the U.S. and expects to submit an investigational new drug (IND) application in the first half of 2024.
- Beam is also advancing lead liver-targeted programs from its collaborations with Pfizer and Apellis.

Sustainable Research Portfolio

- Beam's near-term research and platform investments are focused on specific applications leveraging Beam's *in vivo* editing capabilities in the liver targeting both rare and common genetic disorders, as well as opportunities in hematology and immunology/oncology.
- Enrollment in the company's Phase 1/2 clinical trial of BEAM-201, a multiplex-edited allogeneic CAR-T product candidate, is ongoing for the treatment of relapsed/refractory T-cell acute lymphoblastic leukemia (T-ALL)/T-cell lymphoblastic lymphoma (T-LL). Beam expects to report an initial clinical dataset for BEAM-201 in the second half of 2024.

Cash Position and Updated Operating Runway

As of December 31, 2023, Beam estimates that it had $1.2 billion 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, 2023, and its results of operations for the three months and year ended December 31, 2023. 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, 2023 will enable the company to fund its anticipated operating expenses and capital expenditure requirements into 2027. This expectation assumes anticipated cost savings related to the company's previously announced portfolio prioritization and streamlining of operations and includes funding directed toward reaching each of the key anticipated 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 42 nd Annual J.P. Morgan Healthcare Conference today, Monday, January 8, 2024, 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 42nd 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, AATD, GSD1a, T-ALL/TLL, and our conditioning regimens; our plans, and anticipated timing, to advance our programs, the clinical trial designs and expectations for BEAM-101, BEAM-201, BEAM-301 and BEAM-302; our estimated cash, cash equivalents and marketable securities as of December 31, 2023 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 successfully achieve the benefits of our portfolio prioritization and strategic restructuring; 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 pandemics and other health emergencies, including their 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 initiation and enrollment of, and anticipated timing to advance, 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, 2023; 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, 2022, our Quarterly Report on Form 10-Q for the quarter ended March 31, 2023, our Quarterly Report on Form 10-Q for the quarter ended June 30, 2023, our Quarterly Report on Form 10-Q for the quarter ended September 30, 2023, 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
THRUST Strategic Communications
holly@thrustsc.com

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