Beam Therapeutics Reports Progress Across Ex Vivo and In Vivo Pipeline of Base Editing Therapeutics and Outlines Key Anticipated 2022 Milestones

January 9, 2022

First Subject Anticipated to be Enrolled in BEAM-101 Phase 1/2 Clinical Trial for the Treatment of Sickle Cell Disease in the Second Half of 2022

BEAM-301 Named as Fourth Development Candidate for the Treatment of Glycogen Storage Disease Type Ia

Nomination of Two Additional Development Candidates Anticipated in 2022

Company to Present Pipeline and Business Updates at 40th Annual J.P. Morgan Healthcare Conference on January 10, 2022, at 2:15 p.m. ET

CAMBRIDGE, Mass., Jan. 09, 2022 (GLOBE NEWSWIRE) -- Beam Therapeutics Inc. (Nasdaq: BEAM), a biotechnology company developing precision genetic medicines through base editing, today outlined anticipated 2022 milestones across its *ex vivo* programs targeting editing of hematopoietic stem cells (HSCs) and T cells and *in vivo* programs targeting editing of liver cells leveraging lipid nanoparticles (LNPs) for delivery. Updates include that the company has selected its fourth development candidate and first *in vivo* base editing candidate, BEAM-301, which aims to correct the R83C mutation for the potential treatment of patients with glycogen storage disorder Ia (GSDIa).

"We made significant progress across our base editing portfolio in 2021, which culminated in U.S. Food and Drug Administration clearance of the first investigational new drug application of a base editing therapeutic, BEAM-101. We also further expanded our platform, particularly with LNP delivery of base editors to the liver and our proprietary technology for accelerating LNP delivery to other tissues, including HSCs," said John Evans, chief executive officer of Beam. "We believe 2022 is set to be our most important year yet, with preparations underway to launch the BEACON-101 clinical trial with BEAM-101 for the treatment of sickle cell disease and to complete our transition to becoming a clinical-stage company. We believe we are well positioned today, with four development candidates, a rich pipeline of earlier stage programs, and an industry-leading platform of editing and delivery technologies enabling us to bring forward a new class of precision genetic medicines. None of this would be possible without the commitment of our remarkable team of fearless innovators. We look forward to the year ahead and continuing our work to bring potentially life-changing medicines to as many patients as possible."

***Ex Vivo* HSC Programs**

- BEAM-101 is a patient-specific, autologous HSC investigational therapy, which incorporates base edits that are designed to mimic single nucleotide polymorphisms seen in individuals with hereditary persistence of fetal hemoglobin. BEAM-101 aims to potentially alleviate the effects of mutations causing sickle cell disease (SCD) or beta-thalassemia by leading to increases in fetal hemoglobin, which inhibits hemoglobin S (HbS) polymerization. The BEACON-101 trial is a Phase 1/2 clinical trial designed to assess the safety and efficacy of BEAM-101 for the treatment of SCD. The trial is expected to include an initial "sentinel" cohort of three patients, treated one at a time to confirm successful engraftment, followed by dosing in up to a total of 45 patients. Beam has begun site selection and the institutional review board approval processes for the BEACON-101 trial and plans to enroll the first subject in the second half of 2022.
- BEAM-102 is designed to treat SCD by directly editing the causative HbS point mutation to recreate a naturally occurring normal human hemoglobin variant, HbG-Makassar. The Makassar variant has been reported to have the same function as the more common HbA variant and does not cause SCD. Beam plans to submit an investigational new drug (IND) application for BEAM-102 in the second half of 2022.

***Ex Vivo* T Cell Programs**

- BEAM-201 is a multiplex base edited anti-CD7 CAR-T cell investigational therapy designed to treat relapsed/refractory T cell acute lymphoblastic leukemia, a severe disease affecting children and adults. Beam plans to submit an IND application for BEAM-201 in the second half of 2022.
- Beam plans to nominate a second CAR-T development candidate in 2022.

***In Vivo* LNP Liver-targeting Programs**

- BEAM-301, the company's newest development candidate, is a liver-targeting LNP formulation of base editing reagents designed to correct the R83C mutation. R83C is the most common disease-causing mutation of GSDIa, a life-altering genetic disease with no approved disease-modifying treatments available today. Beam anticipates initiating IND-enabling studies for BEAM-301 in 2022.
- Beam plans to nominate a second liver-targeted development candidate in 2022.

J.P. Morgan Healthcare Conference

Mr. Evans will present Beam's pipeline and business updates during a presentation at the 40 th Annual J.P. Morgan Healthcare Conference on Monday,

January 10, 2022, at 2:15 p.m. ET. 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 enables precise, predictable and efficient single base changes, at targeted genomic sequences, without making double-stranded breaks in the DNA. This enables 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 plans, and anticipated timing, to nominate additional development candidates, initiate IND-enabling studies, and submit IND applications; the therapeutic applications and potential of our technology, including with respect to sickle cell disease, beta-thalassemia, T-ALL, GSDIa, and LNPs; the planned initiation and design of our BEACON-101 clinical trial, including the timing of enrolling the first subject in the trial; our planned presentations at an upcoming conference; 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; 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 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; 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, 2020, our Quarterly Report on Form 10-Q for the quarter ended March 31, 2021, our Quarterly Report on Form 10-Q for the quarter ended June 30, 2021 and our Quarterly Report on Form 10-Q for the quarter ended September 30, 2021, 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:
Chelcie Lister
THRUST Strategic Communications
chelcie@thrustsc.com

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