Beam Therapeutics Enrolls First Patient in BEACON Clinical Trial of BEAM-101 Base Editing Therapy Candidate for the Treatment of Sickle Cell Disease

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BEAM-101 BEACON Trial Represents the First Clinical Trial of a Base Editor in the United States

CAMBRIDGE, Mass., Nov. 14, 2022 (GLOBE NEWSWIRE) -- Beam Therapeutics Inc. (Nasdaq: BEAM), a biotechnology company developing precision genetic medicines through base editing, today announced that the first patient has been enrolled in the company's BEACON trial. BEACON is an open-label, single-arm, multicenter, Phase 1/2 clinical trial designed to evaluate the safety and efficacy of BEAM-101 in adult patients with severe sickle cell disease (SCD).

BEAM-101 is a patient-specific, autologous hematopoietic stem cell (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 SCD by leading to increases in fetal hemoglobin (HbF), which inhibits hemoglobin S (HbS) polymerization. Using base editing, a next-generation form of CRISPR, BEAM-101 in preclinical studies featured high levels of HSC editing (over 90% of alleles edited), high and consistent levels of upregulation of HbF (over 60% of total hemoglobin), and significant reductions in the disease-causing protein HbS (less than 40% of total hemoglobin) – levels that are similar to sickle cell trait carriers, who do not have SCD. Unlike nuclease editors, the BEAM-101 base editor is designed to avoid double stranded breaks during editing, which can result in unwanted chromosomal abnormalities and genotoxic stress.

Following enrollment in the BEACON trial, each patient will undergo a transfusion and mobilization process for HSC retrieval. The cells are then edited, creating an autologous BEAM-101 investigational drug product. Following the drug product manufacturing, patients in the trial will receive pre-treatment conditioning using a standard-of-care chemotherapy regimen, after which the edited cells are transplanted back into the patient. 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.

"The enrollment of the first patient in our BEACON trial is a significant step forward for Beam and for the field of base editing," said John Evans, chief executive officer of Beam. "With the potency and precision of base editing, we believe BEAM-101 could be a best-in-class option for SCD patients with several advantages over other available genetic therapies. We are now focused on activating additional clinical trial sites in the U.S., modifying the BEACON protocol to enable expedited future patient enrollment and endpoint assessment, and finalizing our commercial-ready manufacturing process. We look forward to advancing this program for patients who suffer from the painful and debilitating consequences of SCD."

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: the clinical trial protocol for BEACON; the therapeutic applications and potential of our technology, including with respect to SCD; 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; 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.

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