Beam Therapeutics Announces U.S. FDA Orphan Drug Designation Granted to BEAM-101 for the Treatment of Sickle Cell Disease

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CAMBRIDGE, Mass., June 03, 2025 (GLOBE NEWSWIRE) -- Beam Therapeutics Inc. (Nasdaq: BEAM), a biotechnology company developing precision genetic medicines through base editing, today announced that the United States (U.S.) Food and Drug Administration (FDA) has granted orphan drug designation to BEAM-101, an investigational genetically modified cell therapy for the treatment of sickle cell disease (SCD).

“Sickle cell disease is a devasting disorder that affects approximately 100,000 people in the U.S., leading to anemia, severe pain, stroke and even early death. Receiving orphan drug designation from the FDA emphasizes the importance of new treatment options for this debilitating disease, and our clinical data suggest that BEAM-101, the lead program in our hematology franchise, has the potential to offer a differentiated, best-in-class treatment,” said Amy Simon, M.D., chief medical officer of Beam. “We look forward to continuing to progress our BEACON Phase 1/2 clinical trial of BEAM-101 in patients with severe sickle cell disease and to working closely with the FDA with the goal of bringing BEAM-101 to patients as safely and quickly as possible, embodying our mission of providing lifelong cures to patients suffering from severe diseases.”

The FDA’s orphan drug designation is designed to support the development and evaluation of treatments for rare diseases affecting fewer than 200,000 people in the U.S. The designation comes with potential benefits for the sponsor company, including tax credits for qualified clinical trials, exemption from user fees, and a potential seven years of market exclusivity after approval.

Data from seven patients treated with BEAM-101 in the BEACON clinical trial were presented at the 66th American Society of Hematology (ASH) Annual Meeting and Exposition in December 2024. Updated clinical data have been accepted for presentation at the European Hematology Association (EHA) 2025 Congress in June. Treatment with BEAM-101 demonstrated robust and durable increases in fetal hemoglobin (HbF) and reductions in sickle hemoglobin (HbS), rapid neutrophil and platelet engraftment, and normalized or improved markers of hemolysis. The initial safety profile of BEAM-101 was consistent with busulfan conditioning and autologous hematopoietic stem cell transplantation. Manufactured in Beam’s North Carolina facility, BEAM-101 uses an advanced, largely automated process that has demonstrated consistently high yields and viability, enabling successful BEAM-101 manufacturing for patients in the BEACON clinical trial. Beam expects to dose 30 patients in the ongoing BEACON trial by mid-2025.

About BEAM-101
BEAM-101 is an investigational genetically modified cell therapy for the treatment of severe sickle cell disease (SCD). The one-time therapy consists of autologous CD34+ hematopoietic stem and progenitor cells (HSPCs) that have been base-edited in the promotor regions of the HBG1/2 genes and are administered via a hematopoietic stem cell transplant procedure. The BEAM-101 edit is designed to inhibit the transcriptional repressor BCL11A from binding to the promoter without disrupting BCL11A expression, leading to increased production of non-sickling and anti-sickling fetal hemoglobin (HbF) and thus mimicking the effects of naturally occurring variants seen in hereditary persistence of fetal hemoglobin. HbF is the predominant hemoglobin variant during development and early life. The safety and efficacy of BEAM-101 is being evaluated in the ongoing BEACON Phase 1/2 study, an open-label, single-arm, multicenter trial in adult patients with SCD with severe vaso-occlusive crises (VOCs).

About Sickle Cell Disease
Sickle cell disease, a severe inherited blood disease, is caused by a single point mutation, E6V, in the beta globin gene. This mutation causes the mutated form of sickle hemoglobin (HbS) to aggregate into long, rigid molecules that bend red blood cells into a sickle shape under conditions of low oxygen. Sickled cells obstruct blood vessels and die prematurely, ultimately resulting in anemia, severe pain (crises), infections, stroke, organ failure and early death. Sickle cell disease is the most common inherited blood disorder in the United States, affecting an estimated 100,000 individuals within the United States and approximately eight million people worldwide.

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 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 therapeutic applications and potential of our technology, including with respect to SCD; our plans, and anticipated timing, to advance our programs, including the clinical trial designs and expectations for BEAM-101; our plans to present data at upcoming medical conferences; expectations regarding potential benefits of the orphan drug designation for BEAM-101; 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 advance 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 or 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; our ability to recognize the potential benefits conferred by the orphan drug designation for BEAM-101; 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, 2024, our Quarterly Report on Form 10-Q for the quarter ended March 31, 2025, 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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