Beam Therapeutics Announces U.S. FDA Regenerative Medicine Advanced Therapy (RMAT) Designation Granted to BEAM-101 for the Treatment of Sickle Cell Disease

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CAMBRIDGE, Mass., Aug. 14, 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 Regenerative Medicine Advanced Therapy (RMAT) designation to BEAM-101, an investigational genetically modified cell therapy for the treatment of sickle cell disease (SCD).

“We are thrilled that the FDA has granted RMAT designation to BEAM-101, following orphan drug designation earlier this year, reinforcing its potential as a one-time, best-in-class therapy for severe sickle cell disease,” said Giuseppe Ciaramella, Ph.D., president of Beam Therapeutics. “These designations not only recognize the promise of BEAM-101 but also enable enhanced collaboration with the FDA as we advance toward a BLA filing. With 30 patients now dosed in the BEACON Phase 1/2 trial and additional data expected later this year, we remain focused on delivering a transformative treatment to people living with sickle cell disease.”

The FDA’s RMAT designation is designed to support the development and evaluation of regenerative medicines, including genetic therapies, with the intention of addressing serious or life-threatening diseases that have unmet medical needs. RMAT designation provides opportunities for early interactions with the FDA to discuss potential surrogate or intermediate endpoints to support accelerated approval, organizational commitment from senior staff at the agency, opportunities to participate in novel review and development programs, and the potential for a rolling review and priority review of a product’s future biologics license application.

Updated clinical data from the BEACON Phase 1/2 clinical trial of BEAM-101 were presented at the European Hematology Association (EHA) 2025 Congress in June, providing further demonstration of the strong clinical profile for BEAM-101, as initially established in previously announced data at the 66th American Society of Hematology (ASH) Annual Meeting and Exposition in December 2024. Updated data from 17 patients treated 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 and oxygen delivery. Patients required a median of one mobilization cycle. No VOCs were reported post-engraftment. BEAM-101 is manufactured using an advanced, largely automated process that has demonstrated consistently high yields and viability.

Beam previously announced that the FDA granted orphan drug designation to BEAM-101 in June, and the company plans to present updated data from the BEACON Phase 1/2 trial by the end of 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 (SCD), 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. SCD is the most common inherited blood disorder in the United States (U.S.), affecting an estimated 100,000 individuals within the U.S. 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 design and expectations for BEAM-101; our plans and anticipated timing to present data from ongoing clinical trials; 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; 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 Reports on Form 10-Q, 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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