Beam Therapeutics Announces First Patient Dosed in Phase 1/2 Trial of BEAM-201 in Relapsed, Refractory T-ALL/T-LL

September 5, 2023

BEAM-201 Represents First Quadruplex-edited, Allogeneic CAR-T Cell Therapy Candidate in Clinical-stage Development and First Treatment with a Base Editing Candidate in United States

CAMBRIDGE, Mass., Sept. 05, 2023 (GLOBE NEWSWIRE) -- Beam Therapeutics Inc. (Nasdaq: BEAM), a biotechnology company developing precision genetic medicines through base editing, today announced that in August, the first patient was treated with BEAM-201, a quadruplex-edited allogeneic CAR-T cell investigational therapy. BEAM-201 is being evaluated in a Phase 1/2 clinical study for the treatment of relapsed/refractory T-cell acute lymphoblastic leukemia/T-cell lymphoblastic lymphoma (T-ALL/T-LL), a severe disease affecting children and adults.

“As the first patient dosed with a Beam therapeutic candidate and the first patient in the U.S. to receive a base editing therapeutic, this represents a major milestone for the company, the scientists that made this possible, and the patients we hope to serve,” said John Evans, chief executive officer of Beam. “We believe that the full therapeutic potential of CAR-T therapies, including the ability to utilize an allogeneic source of T cells, will only be unlocked through higher levels of cellular engineering enabled by multiple simultaneous genetic edits. Base editing is especially well-suited to this challenge, as it is designed to deliver highly efficient multiplex edits in cells without the double stranded breaks that can lead to frequent chromosomal rearrangements and loss of cell viability. BEAM-201, to our knowledge the first quadruplex-edited cell therapy candidate in clinical development, is an allogeneic CAR-T cell investigational therapy with the potential to make a substantial impact for patients diagnosed with challenging T-cell cancers who have limited treatment options.”

About the BEAM-201 Phase 1/2 Clinical Trial
The ongoing Phase 1/2 trial of BEAM-201 is a multicenter, open-label study evaluating safety and efficacy in patients with relapsed/refractory T-ALL/T-LL. T-ALL/T-LL is a highly aggressive blood cancer arising from malignant transformation of T cell precursors that has few treatment options.

The primary objectives of the Phase 1 portion of the trial are the assessment of safety, tolerability, and identification of the recommended Phase 2 dose and lymphodepletion regimen. Key safety endpoints for the trial include treatment-emergent and treatment-related adverse events, and key efficacy endpoints include proportion of patients with complete or partial responses, proportion eligible for hematopoietic stem cell transplant, and proportion achieving minimal residual disease negative status. Multiple sites for the Phase 1/2 clinical trial of BEAM-201 are now open for enrollment. For more information, visit clinicaltrials.gov (NCT05885464).

About BEAM-201
BEAM-201 is a quadruplex base-edited, anti-CD7 allogeneic chimeric antigen receptor T cell (CAR-T) under clinical investigation for the treatment of CD7+ relapsed/refractory T-cell acute lymphoblastic leukemia/T-cell lymphoblastic lymphoma (T-ALL/T-LL). Multiplexed base editing is designed to eliminate expression of the CD7, TRAC, PDCD1 and CD52 genes. This approach has the potential to reduce fratricide, graft-versus-host disease and CAR-T cell exhaustion and to enable BEAM-201 cells to evade anti-CD52 lymphodepleting agents and enable use of an allogeneic cell source.

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 therapeutic applications and potential of our technology, including with respect to T-ALL/TLL; our plans, and anticipated timing, to advance our clinical trials and programs, including our trial of BEAM-201; 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 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; 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 June 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.

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