Beam Therapeutics Reports Data Highlighting Optimized Manufacturing Process for BEAM-101, an Investigational Base Editing Therapeutic for Sickle Cell Disease

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MADRID, June 14, 2024 (GLOBE NEWSWIRE) -- Beam Therapeutics Inc. (Nasdaq: BEAM), a biotechnology company developing precision genetic medicines through base editing, today reported data highlighting its optimized, closed and automated manufacturing process for its base-edited CD34+ hematopoietic stem and progenitor cell (HSPC) genetic medicines in a poster presentation at the European Hematology Association (EHA) Hybrid Congress. The optimized process is deployed for the manufacturing of BEAM-101 in the BEACON Phase 1/2 clinical trial in patients with severe sickle cell disease (SCD), and the data include both preclinical and GMP clinical manufacturing experience to date.

“The manufacturing of autologous cell and gene therapies, particularly for sickle cell disease, is complex and has significant implications for product quality as well as the patient experience,” said Giuseppe Ciaramella, Ph.D., president of Beam. “The data presented today at EHA demonstrate that our optimized process for the manufacturing of BEAM-101, which integrates key technologies and automation improvements, has achieved reproducible and robust product yields and viability that meet demanding high-quality standards. The use of base editing technology is an important advantage, as the avoidance of double-stranded breaks, along with the high frequency of productive edits in each cell, have contributed to the meaningfully high editing rates. Ultimately, these results, combined with our impressive preclinical data package, support our belief that BEAM-101 has the potential to be a highly differentiated product by minimizing the number of stem cell collections needed to deliver a patient dose as well as inducing the deepest resolution of sickle cell disease.”

Beam designed its automated CD34+ HSPC process for manufacturing of BEAM-101 to have the flexibility for a wide range of patient-starting material and variable cell numbers, while maintaining robust cell yield and high drug product quality suitable for use in the BEACON Phase 1/2 clinical trial. Today's data highlight the following:

- The integration of key technologies has led to robust and improved process performance.
- Automation improved manufacturing execution, including increased cumulative yield and process consistency, and provided an up to three-fold increase in process capacity while reducing process duration, contamination risk and operator variability.
- Drug product reproducibly met the high viability threshold across 14 development runs using healthy and sickle cell trait donor cells and nine GMP clinical runs using SCD patient cells.
- The use of base editing in an optimized, closed and automated process produced clinical drug product with consistently high CD34+ purity ranging from 84% to 95% and an editing rate ranging from 88% to 94%.

About BEAM-101
BEAM-101 is an investigational genetically modified cell therapy for the treatment of 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.

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 sickle cell disease; our plans, and anticipated timing, to advance our BEAM-101 program; 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 successfully achieve the benefits of our portfolio prioritization and strategic restructuring; 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; 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, 2023, our Quarterly Report on Form 10-Q for the quarter ended March 31, 2024, 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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