Beam Therapeutics Presents First Data Highlighting Base Editing Program for Glycogen Storage Disease Type Ia at AASLD

November 13, 2020

Novel Base Editors for Two Most Common GSDIa Mutations Demonstrate Significantly Higher Levels of In Vivo Mutation Correction than Required to Restore Glucose Homeostasis

Previously Presented Data from Alpha-1 Program to be Reviewed in Encore Presentation

CAMBRIDGE, Mass., Nov. 13, 2020 (GLOBE NEWSWIRE) -- Beam Therapeutics Inc. (Nasdaq: BEAM), a biotechnology company developing precision genetic medicines through base editing, today announced that the company will present preclinical data from its liver-focused programs, including the first data highlighting its novel base-editing strategy for correcting disease-causing mutations underlying Glycogen Storage Disease Type Ia (GSDIa), during the American Association for the Study of Liver Diseases (AASLD) The Liver Meeting Digital Experience being held virtually November 13-16, 2020.

GSDIa, also known as Von Gierke disease, is an inborn disorder of glucose metabolism caused by mutations in the G6PC gene that disrupt a key enzyme, Glucose-6- Phosphatase (G6Pase), which is involved in glucose homeostasis. This disruption results in low blood glucose levels that can be fatal if patients do not adhere to a strict regimen of slow-release forms of glucose, administered every one to four hours (including overnight). There are currently no pharmacological therapies approved for patients with GSDIa. Beam has engineered novel adenine base editors (ABE) that, in preclinical models, have achieved high levels of precise correction of the two most prevalent GSD1a mutations, R83C and Q347X, in both *in vitro* and *in vivo* settings.

“Base editing represents an exciting new opportunity for the treatment of serious diseases, including GSDIa, where patients are in need of a disease-modifying, life-long treatment,” said Giuseppe Ciaramella, Ph.D., president and chief scientific officer of Beam. “These first preclinical data from our GSDIa program demonstrate significant levels of precise correction of the disease-causing R83C and Q347X point mutations, well above the level that we believe is needed to have a clinically relevant treatment effect for patients with GSD1a. We look forward to presenting these data, as well as our encore presentation of our Alpha-1 data from ASGCT, as we continue to advance our liver base editing programs toward the clinic.”

Details of the GSDIa presentation are as follows:

Title: Base-Editing as a Therapeutic Approach for the Direct Correction of Disease-Causing Mutations Underlying Glycogen Storage Disease Type Ia
Publication Number: 0589
Session Title: Genomics and Precision Medicine
Data Summary: Beam’s approach to treating patients with GSDIa is to deliver an ABE via lipid nanoparticle (LNP) to the liver to repair either the R83C or the Q347X mutations in G6PC. It is estimated that these two-point mutations account for 900 and 500 patients, respectively, in the United States, representing approximately 60% of all GSDIa patients. Animal studies suggest that a critical therapeutic threshold of approximately 11% of normal G6Pase activity in liver cells is sufficient to restore fasting glucose; however, this level must be maintained in order to preserve glucose control and alleviate other serious, and potentially fatal, GSDIa symptoms. *In vivo* correction of both mutations by ABEs was observed in the livers of two strains of transgenic mice, each carrying one of the two G6PC mutations. Next-generation sequencing data from whole liver extracts reveal significant correction for both R83C and Q347X, with nearly 40% and approximately 70% A-to-G conversion efficiency, respectively, of each mutation back to the normal gene sequence. These significant levels of mutation correction greatly surpass those expected to restore glucose homeostasis, and functional studies are ongoing to correlate pathophysiology to extent of mutation correction by base-editing. Further, these levels of *in vivo* correction for GSDIa by base-editing are achieved without creation of double-stranded breaks. In total, these data support base-editing technology as a promising approach for precise correction of causative mutations in GSDIa.

Beam will also report data during an oral presentation at AASLD from its Alpha-1 Antitrypsin Deficiency (Alpha-1) program, which were previously presented at American Society of Gene & Cell Therapy 2020 Annual Meeting. Details of the Alpha-1 presentation are as follows:

Title: Evaluation of Adenine Base Editing as a Potential Treatment for Alpha-1 Antitrypsin Deficiency
Publication Number: 0032
Session Title: Parallel 3: Metabolic and Genetic Diseases
Session Broadcast Date and Time: Saturday, November 14, 2020, 2:00 p.m. ET

About Beam Therapeutics
Beam Therapeutics (Nasdaq: BEAM) is a biotechnology company developing precision genetic medicines through the use of base editing. Beam’s proprietary base editors create precise, predictable and efficient single base changes, at targeted genomic sequences, without making double-stranded breaks in the DNA. This enables 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. For more information, visit www.beamtx.com.

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: our plans for scientific

publications; our plans to advance our liver base editing programs toward the clinic; the expected levels of GSDIa mutation correction that are required to be clinically relevant and disease-modifying in humans; and the therapeutic applications and potential of our technology, including our ability to develop precision genetic medicines for patients through base editing. Each forward-looking statement is subject to 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 potentially 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; that preclinical testing of our product candidates and preliminary or interim data from preclinical and clinical trials may not be predictive of the results or success of ongoing or later clinical trials; that enrollment 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 heading “Risk Factors” in our Annual Report on Form 10-K for the year ended December 31, 2019, our Quarterly Report on Form 10-Q for the quarters ended March 31, 2020, June 30, 2020, and September 30, 2020, and in any subsequent filings with the Securities and Exchange Commission. These forward-looking statements (except as otherwise noted) 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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