Beam Therapeutics Announces Preclinical Data Highlighting Base Editing Approach to Correct a Glycogen Storage Disease Type Ia Disease-Causing Mutation

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Findings Show Base Editing Leads to Elimination of Disease Phenotype in an In Vivo Model

CAMBRIDGE, Mass., Oct. 19, 2021 (GLOBE NEWSWIRE) -- Beam Therapeutics Inc. (Nasdaq: BEAM), a biotechnology company developing precision genetic medicines through base editing, today reported new preclinical data demonstrating the ability of its liver-targeted base editing approach to directly correct R83C, one of the primary disease-causing mutations of glycogen storage disease type Ia (GSDIa). The data will be presented at the European Society of Gene and Cell Therapy (ESGCT) 2021 Virtual Congress in an oral presentation at 9:45AM (EST) / 3:45PM (CEST) on Wednesday, October 20, 2021, during the Liver & Metabolic Disease I Session 3a.

"The data we will present at ESGCT highlight the continued innovation of our *in viv*o, liver-targeted base editing approach for GSDIa, which aims to directly correct R83C, a highly prevalent disease-causing mutation in this patient population," said Giuseppe Ciaramella, Ph.D., president and chief scientific officer of Beam. "These findings showcase the exceptional work of our team of scientists, who created a novel, humanized GSDIa R83C mouse model. When treated with our base editor, we directly corrected the human gene by editing a single base in the gene, without creating double-stranded breaks in DNA, and restored normal glucose metabolism in these animals. These data demonstrate robust preclinical proof of concept for the treatment of GSDIa and add to the significant collection of research findings we have generated supporting the promise of base editing to address a wide range of diseases."

GSDIa is an autosomal recessive disorder caused by mutations in the G6PC gene that disrupt a key enzyme, glucose-6- phosphatase (G6Pase), which is involved in maintaining glucose homeostasis. Inhibition of G6Pase activity results in low fasting blood glucose levels that can be fatal. To mitigate fasting hypoglycemia, GSDIa patients must adhere to a strict regimen of slow-release forms of glucose, administered every one to four hours, including overnight.

Beam's approach to treating GSDIa is to deliver an adenine base editor (ABE) via lipid nanoparticle (LNP) to the liver to repair the G6PC-p.R83C mutation. To evaluate its approach, Beam created a novel, humanized R83C knockout mouse model (huR83C), mimicking the abnormal metabolic phenotype of human GSDIa and collaborated with experts at the NIH to characterize the phenotype of these animals.

The results demonstrate that newborn huR83C mice treated with Beam's LNP-delivered ABE exhibit normal growth to the end of the study at three weeks of age without any hypoglycemia-induced seizures. In contrast, homozygous animals are unable to survive soon after birth in the absence of glucose supplementation. In addition, Beam observed editing efficiencies up to approximately 60% by next-generation sequencing of DNA isolated from the whole liver. Of note, even narrow gains in base editing efficiency are associated with significant restoration of G6Pase activity and normal metabolic function. Published studies suggest that a critical therapeutic threshold of approximately11% of normal G6Pase activity in the liver is sufficient to mitigate fasting hypoglycemia in animal models of GSDIa.

These findings support the potential of base editing to correct disease-causing mutations in GSDIa, and Beam plans to advance evaluation of this research program through additional preclinical studies.

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 enables 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.

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 planned base editing data presentations at upcoming scientific conferences; and the therapeutic applications and potential of our technology, including for GSDIa, and our ability to develop life-long, curative, 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 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 headings "Risk Factors Summary" and "Risk Factors" in our Annual Report on Form 10-K for the year ended December 31, 2020, our Quarterly Report on Form 10-Q for the quarter ended March 31, 2021, our Quarterly Report on Form 10-Q for the quarter ended June 30, 2021, 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.

Contacts:

Investors:
Chelcie Lister
THRUST Strategic Communications
chelcie@thrustsc.com

Media:
Dan Budwick
1AB
dan@1abmedia.com