Beam Therapeutics to Present First Data Highlighting Base Editing Program for Alpha-1 Antitrypsin Deficiency at 23rd ASGCT Annual Meeting

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Preclinical Data Demonstrate Efficient Base Editing Leading to Durable Levels of Precise Gene Correction In Vivo

CAMBRIDGE, Mass., April 28, 2020 (GLOBE NEWSWIRE) -- Beam Therapeutics Inc. (Nasdaq: BEAM), a biotechnology company developing precision genetic medicines through base editing, today announced preclinical data showcasing the potential of its novel base editing approach for the treatment of alpha-1 antitrypsin deficiency (Alpha-1) liver and lung diseases. The data will be presented in oral and poster sessions during the 23rd American Society of Gene and Cell Therapy (ASGCT) Annual Meeting, which will be hosted virtually May 12-15, 2020. In addition, Beam will highlight data from its Alpha-1 program in a pre-meeting workshop presentation on Monday, May 11, 2020 from 3:50 p.m. – 4:10 p.m. ET.

Alpha-1 is a rare, inherited genetic disorder that can cause progressive lung and liver disease. It is most commonly caused by a G-to-A point mutation – referred to as the PiZ allele – within the SERPINA1 gene, which produces alpha-1 antitrypsin (A1AT) protein. In healthy individuals, the A1AT protein is secreted from the liver and circulates to protect the lungs from damage. In individuals with the mutation, A1AT is misfolded, preventing secretion and resulting in damaging build-up in the liver, as well as loss of its protective function in the lungs. There are currently no curative treatments for patients with Alpha-1.

“Base editing represents a new frontier in the treatment of serious diseases, including Alpha-1. Directly and permanently correcting the PiZ mutation could potentially deliver a one-time therapy for Alpha-1 patients that addresses both the liver and lung pathologies in this disease,” said John Evans, chief executive officer of Beam. “These are the first data to be reported from our Alpha-1 program, providing both *in vitro* and *in vivo* proof of concept for base editing to correct this disease. Our base editors demonstrated high efficiency and durability of correction, as well as measurable restoration of A1AT levels and functional activity, validating their potential as a treatment for Alpha-1 patients. We look forward to presenting these and additional data generated since abstract submission at ASGCT.”

Poster Presentation: *Use of Adenine Base Editors to Precisely Correct the Disease-Causing PiZ Mutation in Alpha-1 Antitrypsin Deficiency*
Session: Metabolic, Storage, Endocrine, Liver and Gastrointestinal Diseases
Date and Time: Wednesday, May 13, 2020, 5:30 p.m. – 6:30 p.m. ET

Study Approach and Key Findings: Beam’s program to address Alpha-1 utilizes its adenine base editors (ABEs) to enable the programmable conversion of A-to-T to G-to-C base pairs and precisely correct the disease-causing PiZ mutation. For this study, Beam engineered novel ABEs and guide RNAs capable of correcting the PiZ mutation, and then applied a proprietary non-viral lipid nanoparticle formulation to deliver the optimized reagents to the livers of PiZ transgenic mice. This direct editing approach resulted in significant levels of precise correction of the PiZ mutation, which were maintained through the duration of the multi-month experiment. Further, the precise correction was associated with decreased A1AT globule burden in the liver and with a beneficial increase in serum A1AT levels and elastase inhibitory capacity. These data indicate the potential for base editing to treat both lung and liver manifestations of Alpha-1, supporting the therapeutic opportunity for base editing in treating this disease.

Oral Presentation: *Base Editing of an Allosteric Site within SERPINA1 Yields Improved Phenotypes in Models of Alpha-1 Antitrypsin Deficiency*
Session: Gene Therapy for Inborn Errors of Metabolism: New Approaches
Date and Time: Friday, May 15, 2020, 11:30 a.m. – 11:45 a.m. ET

Study Approach and Key Findings: An exploratory experiment was conducted as part of an academic collaboration with Boston University, the presenter of the data, to assess a compensatory base editing approach for Alpha-1. Beam’s cytosine base editor (BE4) was used to introduce a C-to-T transition mutation within SERPINA1 to generate an allosteric compensatory mutation (M374I) within the A1AT protein. This approach resulted in an increase in serum A1AT concentration and serum elastase inhibitory capacity, as well as a corresponding decrease in A1AT globule burden, as assessed by PAS-D staining, validating Beam’s platform technology and the biology of the model.

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 focused on 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. Investors are cautioned not to place undue reliance on these forward-looking statements, including statements about our plans for scientific publications, the expected timing of filing INDs applications and the therapeutic applications of our technology. 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. Applicable risks and uncertainties include the risks and uncertainties, among other things, regarding: the success in development and potential commercialization of our product candidates; our ability to obtain, maintain and enforce patent and other intellectual property protection for our product candidates; whether preclinical testing of our product candidates and preliminary or interim data from preclinical and clinical trials will be predictive of the results or success of ongoing or later clinical trials; that enrollment of clinical trials may take longer than expected; that our product candidates will experience manufacturing or supply interruptions or failures; that we will be unable to successfully

initiate or complete the preclinical and clinical development and eventual commercialization of our product candidates; that the development and commercialization of our product candidates will take longer or cost more than planned; and the other risks and uncertainties identified under the heading “Risk Factors” and in our Annual Reports on Form 10-K for the year ended December 31, 2019 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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