Beam Therapeutics to Present Updated Data Highlighting Base Editing Programs for Sickle Cell Disease at 23rd ASGCT Annual Meeting

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Data Further Validate Two Novel Base Editing Approaches Leveraging Naturally Occurring Biology to Correct Sickle Cell Mutation

CAMBRIDGE, Mass., April 28, 2020 (GLOBE NEWSWIRE) -- Beam Therapeutics Inc. (Nasdaq: BEAM), a biotechnology company developing precision genetic medicines through base editing, today announced that updated preclinical data, showcasing the potential of its gene editing programs for the treatment of sickle cell disease, will be presented in oral and poster sessions during the 23rd American Society of Gene and Cell Therapy (ASGCT) Annual Meeting, which will be held virtually May 12-15, 2020.

Sickle cell disease, the most common inherited blood disorder in the United States, is caused by a single point mutation in the beta globin gene, which results in the formation of sickle-globin, a component of hemoglobin S (HbS). There are no approved curative treatments for sickle cell disease today. Beam is pursuing two base editing programs to address sickle cell disease, both of which install naturally occurring mutations known to diminish or eliminate pathology associated with the disease in humans.

"We have continued to advance our base editing programs to treat the underlying cause of sickle cell disease, starting with new data from our HPFH program that show meaningful levels in editing and upregulation of fetal hemoglobin, beyond what may be necessary to drive a therapeutic effect for this severe disease, as well as replication of our long-term engraftment data," said Giuseppe Ciaramella, Ph.D., president and chief scientific officer of Beam. "In addition, we are excited to share more data from our base editing program directly converting the causative HbS point mutation to a normal human variant, Hb-G Makassar, which shows unprecedented levels of direct correction in patient-derived cells. This program represents an entirely new paradigm to treating sickle cell disease by directly editing and, for the first time, eliminating the HbS mutation. We look forward to presenting these findings, as well as additional data from our Makassar variant study, at ASGCT."

Oral Presentation: *Base Editing of Gamma Globin Gene Promoters Generates Durable Expression of Fetal Hemoglobin for the Treatment of Sickle Cell Disease*
Session: HSPC Gene Therapies for Hemoglobin Disorders
Date and Time: Wednesday, May 13, 2020, 4:15 p.m. – 4:30 p.m. ET

Background: Fetal hemoglobin (HbF), which is involved in transporting oxygen to a human fetus, is normally silenced after birth. Beam is developing a base editing program for the precise installation of naturally occurring mutations that cause HbF to continue expressing in adulthood, a condition known as hereditary persistence of fetal hemoglobin (HPFH), designed to provide a disease-modifying treatment for multiple hemoglobinopathies, including sickle cell disease.

Study Approach and Key Findings:

- Using its adenine base editors (ABEs), Beam's HPFH program to treat sickle cell disease aims to recreate single base changes in the gamma globin gene promoters (HBG1 and HBG2) that disrupt repressor binding, leading to increased expression of HbF.
- After sorting human erythroid cells from the bone marrow of mice 16 weeks post-engraftment, Beam observed an increase in gamma globin levels of greater than 65% compared to unedited cells, which showed less than 1.5%. Beam was able to replicate these findings with a second donor at 18 weeks post-engraftment, achieving similar results.
- In addition, CD34+ cells from a homozygous sickle patient were successfully edited, showing greater than 60% HbF levels with a concomitant decrease in HbS levels *in vitro*.
- These findings demonstrate that *ex vivo* delivery of ABEs achieved precise editing, resulting in long-term engraftment and therapeutically relevant increases in gamma globin protein expression *in vivo*.

Poster Presentation: *A Novel Base Editing Approach to Directly Edit the Causative Mutation in Sickle Cell Disease*
Session: Hematologic and Immunologic Diseases
Date and Time: Wednesday, May 13, 2020, 5:30 p.m. – 6:30 p.m. ET

Background: Beam is working on a second base editing program to treat sickle cell disease by directly editing the causative HbS point mutation into an asymptomatic, naturally occurring globin variant, HbG-Makassar. The Makassar variant does not cause hemoglobin to polymerize and red cells to sickle. Preclinical data suggest that direct editing to HbG-Makassar could eliminate the sickle protein, representing a promising approach to treating sickle cell disease.

Study Approach and Key Findings:

- Using its ABE platform to edit CD34+ hematopoietic stem and progenitor cells (HSPCs) isolated from donors with the sickle cell trait, Beam achieved editing levels of the HbS mutation of greater than 70% following *in vitro* erythroid differentiation (IVED).

- Beam subsequently developed a novel, ultra-high-performance liquid chromatography assay to detect HbG-Makassar, which showed conversion of sickle HbS to HbG-Makassar at levels near 70% with a simultaneous reduction of HbS globin to 30% of control levels in edited IVED cells from a homozygous SCD patient.
- These findings represent therapeutic levels of correction and support advancement of this program to potentially address the underlying genetic cause of sickle cell disease.

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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