Beam Therapeutics Presents Preclinical Data for Complementary Base Editing Approaches for Hemoglobinopathies at ASH 2019

December 8, 2019

HPFH program achieves in vivo proof of concept of long term engraftment with >90% base editing maintained after 16 weeks

Makassar program demonstrates up to 70% direct editing of the E6V sickle cell point mutation

CAMBRIDGE, Mass. – December 8, 2019 – Beam Therapeutics, a biotechnology company developing precision genetic medicines through base editing, today announced new preclinical data for the company's programs addressing beta-thalassemia and sickle cell disease at the 61st Annual American Society of Hematology (ASH) Annual Meeting in Orlando, Fla.

Beam is pursuing the application of base editing, its proprietary CRISPR-based technology that can make precise edits to single base pairs in DNA and RNA. The company has a pipeline of programs in numerous serious genetic diseases, including sickle cell disease and beta-thalassemia, which are debilitating non-malignant blood disorders that lack effective, disease-modifying treatments.

"Base editing increases the precision, control, and efficiency of gene editing, while avoiding the creation of double-stranded DNA breaks. For the first time, at ASH today we presented data showing our base editing programs in hemoglobinopathies, including the first in vivo proof of concept for long term engraftment of base edited CD34 cells," said Giuseppe Ciaramella, Ph.D., chief scientific officer. "Building on the insights of clinical genetics, we believe that using base editors to precisely recreate these naturally-occurring genetic variants will enable the best possible outcomes for patients suffering from severe hemoglobinopathies. Furthermore, establishing preclinical proof of concept for base editing CD34 cells opens up the potential to expand our base editing pipeline to target other severe hematologic disorders."

Beam presented data at ASH on its two complementary base editing approaches that recreate hereditary persistence of fetal hemoglobin (HPFH) or directly edit the sickle-causing mutation to install the naturally-occurring, asymptomatic Hb G-Makassar variant.

Using a different strategy from other gene editing programs for hemoglobinopathies, in the HPFH approach, base editing is used to recreate single base changes in the regulatory region of both gamma globin genes (HBG1 and HBG2) that disrupt repressor binding and lead to increased expression of fetal hemoglobin (HbF). Beta-thalassemia or sickle cell disease patients naturally harboring these variants are often asymptomatic or experience a milder form of the disease. Beam demonstrated that editing followed by in vitro erythroid differentiation of CD34+ cells from both healthy donors and sickle trait donors led to HbF levels of greater than 60%, which is expected to be clinically relevant. Additionally, the company demonstrated that edited CD34+ cells from a healthy donor engraft with high chimerism and maintain >90% editing after 16 weeks in immunocompromised NBSGW mice.

In parallel, Beam is using base editors to directly convert the sickle cell disease-causing point mutation (E6V) into an asymptomatic, naturally-occurring variant (E6A), also known as Hb G-Makassar. This hemoglobin variant has been observed in individuals who do not have polymerization (sickling) and are otherwise asymptomatic. Beam demonstrated that its base editor variants can reach up to 70% direct correction of the HbS in sickle cell disease patient-derived fibroblasts.

Presentation details:

Title: Complementary base editing approaches for the treatment of sickle cell disease and

beta-thalassemia

Date & Time: Sunday, December 8, 2019, 6:00-8:00 p.m. ET

Poster Session: 801 – Gene Therapy and Transfer: Poster II

Location: Orange County Convention Center, Hall B

Presenter: Ling Lin, Ph.D., senior scientist, Beam Therapeutics

About Beam Therapeutics

Beam Therapeutics is developing precision genetic medicines through base editing. Founded by leading scientists in CRISPR gene editing, Beam is pursuing therapies for serious diseases using its proprietary base editing technology, which can make precise edits to single base pairs in DNA and RNA. Beam is headquartered in Cambridge, Massachusetts. For additional information, visit www.BeamTx.com.

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