A METHOD TO CURE SICKLE CELL DISEASE

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PATENT STATUS

Country	Type	Number	Dated	Case
United States Of America	Published Application	20210155927	05/27/2021	2018-076
European Patent Office	Published Application	3773637 A0	02/17/2021	2018-076

BRIEF DESCRIPTION

Sickle cell disease is an inherited recessive disease, caused by a single nucleotide polymorphism in Beta-globin (HBB). The modified hemoglobin causes normally round red blood cells to take on a sticky, sickle-shaped form. Sickle red blood cells clog blood vessels, causing acute pain “crises” and vasculopathy. Additional complications and consequences associated with sickle cell disease include organ damage, organ failure, increased risk of stroke, pulmonary hypertension, acute chest syndrome (ACS), and decreased lifespan. There is no widely available cure for sickle cell disease. Treatments include allogeneic bone marrow transplants, which can be risky and limited by donor availability.

UC Berkeley researchers and others have created a method of modifying a globin gene in the genome of a hematopoietic stem/progenitor cell (HSPCs) by obtaining HSPCs from an individual with a globin gene having a sickle cell disease (SCD)-associated single nucleotide polymorphism (SNP) to generate an in vitro population of CD34+ HSPCs and then contacting the in vitro population with a genome editing composition, as described in further detail below.

SUGGESTED USES

» therepeutic treatment of sickle cell disease

CONTACT

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INVENTORS

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OTHER INFORMATION

CATEGORIZED AS

» **Medical**

» Disease: Blood and Lymphatic System

» Gene Therapy

» Therapeutics

RELATED CASES

2018-076-0

ADDITIONAL TECHNOLOGIES BY THESE INVENTORS

- Methods And Reagents To Use Cas9 Rnp For Correcting The Hemoglobin Sickle Cell Mutation
- COMPOSITIONS AND METHODS FOR ENHANCED GENOME EDITING
- HDR Reporter Cell Line
- Improvements to Cas9-Mediated Mutation