METHODS AND REAGENTS TO USE CAS9 RNP FOR CORRECTING THE HEMOGLOBIN SICKLE CELL MUTATION

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BRIEF DESCRIPTION

Sickle cell anemia affects millions of people worldwide, and about 100,000 Americans. The disease is caused by a point mutation in the ß-globin gene that alters one amino acid and causes hemoglobin to polymerize. This damages the red blood cell, and can result in hemolytic anemia, vascular damage, and progressive organ damage. While sickle cell disease can be cured by allogeneic hematopoietic cell transplantation, the therapy is risky and there are not many suitable donors. Furthermore, it is difficult to predict disease progression, meaning that this curative therapy cannot be implemented early on.

Researchers at UC Berkeley have developed a system using CRISPR/Cas9 gene editing for a safer therapeutic approach that could cure any patient with sickle cell disease. By using patients' own stem cells, the need for a donor is eliminated.

SUGGESTED USES

- Autologous bone marrow transplant to cure sickle cell disease
- Correct mutations for other diseases by editing hematopoietic stem cells
- Develop preclinical models for research

ADVANTAGES

- Uses patient cells and eliminates need for a donor
- Safer than conventional therapy
- Applicable to any hematopoietic disease

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INVENTORS

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OTHER INFORMATION

KEYWORDS

Cas9, sickle cell anemia, hematopoietic, therapeutic, stem cell

CATEGORIZED AS

- **Biotechnology**
  - Health
- **Medical**
  - Disease: Genetic Diseases and Dysmorphic Syndromes
  - Gene Therapy
  - Therapeutics

RELATED CASES

2016-002-0

ADDITIONAL TECHNOLOGIES BY THESE INVENTORS

- A Method To Cure Sickle Cell Disease
- COMPOSITIONS AND METHODS FOR ENHANCED GENOME EDITING
- HDR Reporter Cell Line
- Improvements to Cas9-Mediated Mutation