Gene Editing: An Improved Methodology For Homology Directed Repair In Cells

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BACKGROUND

Gene editing in cells involves the use of sequence-specific nucleases that generate double-strand DNA breaks (DSBs) at specifically targeted sites in the genome. The DSBs are then repaired by non-homologous end joining (NHEJ) or, much less efficiently, by homology directed repair (HDR). In NHEJ, the two free DNA ends are joined together. This commonly results in small DNA deletions or insertions that inactivate the target gene. In HDR, the DSB is repaired by a mechanism that converts the DNA into the specific sequence that is provided by a DNA donor template. Thus, HDR enables genes to be modified or inserted at specifically desired sites. The main drawback to HDR is the low efficiency of the process.

TECHNOLOGY DESCRIPTION

Researchers at UC San Diego have developed a method to increase the efficiency of homology directed repair (HDR). This method uses CRISPR-Cas9 technology to generate the DNA double-strand breaks, and then employs a novel approach for HDR. In some instances, this method also decreases the amount of cell death that occurs during the HDR process.

APPLICATIONS

The invention has wide applications including use in treatment of human genetic diseases as well as performing genetic engineering on cells or organisms with the goal of producing better pharmaceuticals.

ADVANTAGES

This new invention has been found to increase the HDR efficiency by 2- to 15-fold

STATE OF DEVELOPMENT

A working research model.

INTELLECTUAL PROPERTY INFO

This technology is patent pending and available for licensing and/or research sponsorship.

PATENT STATUS

Country	Type	Number	Dated	Case
Patent Cooperation Treaty	Published Application	WO 2020/036653	02/20/2020	2018-298

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OTHER INFORMATION

KEYWORDS

Gene editing, CRISPR-Cas9, non-homologous end joining, homology directed repair, double-strand DNA breaks, DNA repair

CATEGORIZED AS

- **Biotechnology**
  - Genomics
- **Medical**
  - Gene Therapy
  - Research Tools
- **Research Tools**
  - Nucleic Acids/DNA/RNA

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