COMPOSITIONS AND METHODS FOR ENHANCED GENOME EDITING

Tech ID: 25476 / UC Case 2016-044-0

PATENT STATUS

Country	Type	Number	Dated	Case
United States Of America	Issued Patent	11,427,837	08/30/2022	2016-044

BRIEF DESCRIPTION

Genome editing holds great promise for fundamental discovery, treatment of genetic diseases, and prophylactic treatment. Gene knockouts can be generated using a genome editing endonuclease (e.g., a zinc finger nuclease (ZFN), a transcription activator-like effector nuclease (TALEN), a CRISPR/Cas protein: guide RNA, and the like) to introduce a site-specific double strand break (DSB) within a gene of interest. Clones can be screened for those in which one or more alleles have been repaired in an error-prone fashion to disrupt the open reading frame. However, genome editing reagents can have differential activities, for example variable knockout efficiency stemming from the use of different CRISPR guide RNAs. Thus, there is a need for methods and compositions for increasing the frequency of disrupting mutations (e.g., indels) that can be produced when using targeted genome editing nucleases.

UC Berkeley researchers have discovered a simple way to increase the frequency of the generation of indels gene editing reagents by adding non-homologous DNA to the genome targeting composition (e.g., zinc finger nuclease, TALEN nuclease fusion protein, CRISPR/Cas endonuclease). This approach greatly increases the frequency of knockout alleles, thereby enabling the easy generation of homozygous knockout cell lines and organisms, as well as improving the efficiency of knockout screens.

SUGGESTED USES

- Precision gene editing in therapeutic contexts
- Introduction of desired traits in crops
- Generation of genetically modified organisms
- Isogenic cell line generation

ADVANTAGES

- High efficiency (knockout can be increased to nearly 100%)
- Rescue underperforming gene editing reagents
- Generalizable to multiple cell/organism contexts

ADDITIONAL TECHNOLOGIES BY THESE INVENTORS

- Methods And Reagents To Use Cas9 Rnp For Correcting The Hemoglobin Sickle Cell Mutation
- A Method To Cure Sickle Cell Disease
- HDR Reporter Cell Line
- Improvements to Cas9-Mediated Mutation

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OTHER INFORMATION

KEYWORDS

Cas9, targeted gene repair, genetic engineering, gene editing, CRISPR, gene knockout

CATEGORIZED AS

- **Medical**
  - Gene Therapy
  - Research Tools
  - Therapeutics

RELATED CASES

2016-044-0

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