TARGETED NUCLEIC ACID EDITING VIA CAS9-DEAMINASE FUSION PROTEINS

Tech ID: 34871 / UC Case 2015-054-0

PATENT STATUS

Country	Type	Number	Dated	Case
United States Of America	Issued Patent	10,392,607	08/27/2019	2015-054

BRIEF DESCRIPTION

Traditional genome editing relying on double-stranded DNA breaks (DSBs) can cause unintended indels, translocation events, and cytotoxic responses in target cells. To overcome these limitations, a precise site-specific modification technology is required that operates without inducing double-stranded DNA cleavage. Developed by UC Berkeley researchers, this technology utilizes engineered fusion proteins comprising a Cas domain (such as a Cas9 nickase or dCas9) integrated with a nucleic acid-editing enzyme domain, such as a deaminase. The molecular mechanism leverages the RNA-guided targeted binding capability of Cas9 to localize the enzymatic editing domain to a specific genomic locus, facilitating direct nucleotide alterations (e.g., cytidine or adenosine conversions) without introducing double-stranded cuts. This system delivers high-precision target modification, significantly reduces off-target insertion/deletion mutations, and improves cell viability during genomic modifications compared to conventional double-strand cleavage mechanisms.

SUGGESTED USES

- Gene therapy for therapeutic correction of single-nucleotide genetic disorders in human cells
- Functional genomics and targeted gene disruption or regulation in biomedical research
- Development of genetically engineered crop varieties with specific agronomic traits
- Generation of cellular and animal disease models for high-throughput therapeutic screening

ADVANTAGES

- Enables precise nucleotide modification without introducing double-stranded DNA breaks
- Significantly lower rate of unintended insertion/deletion (indel) mutations relative to standard double-strand breaking enzymes
- High site-specificity driven by programmable RNA-guided Cas domains
- Broad applicability across eukaryotic, prokaryotic, and viral host systems

RELATED MATERIALS

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INVENTORS

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OTHER INFORMATION

CATEGORIZED AS

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

RELATED CASES

2015-054-0

ADDITIONAL TECHNOLOGIES BY THESE INVENTORS

- Next-Generation Lipid Nanoparticles for Precision Nucleic Acid Delivery
- COMPOSITIONS AND METHODS FOR IDENTIFYING HOST CELL TARGET PROTEINS FOR TREATING RNA VIRUS INFECTIONS
- Genome Editing via LNP-Based Delivery of Efficient and Stable CRISPR-Cas Editors
- Tissue-Specific Genome Engineering Using CRISPR-Cas9
- Type III CRISPR-Cas System for Robust RNA Knockdown and Imaging in Eukaryotes
- Cas9 Variants With Altered DNA Cleaving Activity
- Cas12-mediated DNA Detection Reporter Molecules
- Improved guide RNA and Protein Design for CasX-based Gene Editing Platform
- Compositions and Methods for Delivering Molecular Cargo to Cells
- Cas13a/C2c2 - A Dual Function Programmable RNA Endoribonuclease
- Miniature Type VI CRISPR-Cas Systems and Methods of Use
- RNA-directed Cleavage and Modification of DNA using CasY (CRISPR-CasY)
- Generation of Chimeric RNA with Type III CRISPR-Cas
- CasX Nickase Designs, Tans Cleavage Designs & Structure
- Methods and Compositions for Modifying a single stranded Target Nucleic Acid
- Genetically Encoded, Biologically Responsive, And Programmable Bispecific Aptames As Therapeutic Protein Degraders
- A Dual-RNA Guided CasZ Gene Editing Technology
- A Protein Inhibitor Of Cas9
- RNA-directed Cleavage and Modification of DNA using CasX (CRISPR-CasX)
- Compositions and Methods for Genome Editing
- IS110 and IS1111 Family RNA-Guided Transposons
- Variant Cas12a Protein Compositions and Methods of Use
- In Vitro and In Vivo Genome Editing by LNP Delivery of CRISPR Ribonucleoprotein
- CRISPR CASY COMPOSITIONS AND METHODS OF USE
- Single Conjugative Vector for Genome Editing by RNA-guided Transposition
- CRISPR-CAS EFFECTOR POLYPEPTIDES AND METHODS OF USE THEREOF
- Compositions and Methods for VIPR-Based Nucleic Acid Targeting
- Precision-Engineered Cas12a: Eliminating Collateral DNA Cleavage for Next-Generation Genome Editing
- Selective Cell Elimination using RNA-guided Chromatin Shredding
- Methods Of Use Of Cas12L/CasLambda In Plants
- Type V CRISPR/CAS Effector Proteins for Cleaving ssDNA and Detecting Target DNA
- THERMOSTABLE RNA-GUIDED ENDONUCLEASES AND METHODS OF USE THEREOF (GeoCas9)
- Variant TnpB and wRNA Proteins
- Efficient Site-Specific Integration Of New Genetic Information Into Human Cells
- Class 2 CRISPR/Cas COMPOSITIONS AND METHODS OF USE
- Compositions and Methods of Use for Variant Csy4 Endoribonucleases
- Immune Cell-Mediated Intercellular Delivery Of Biomolecules
- Methods and Compositions for Controlling Gene Expression by RNA Processing

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