A DUAL-RNA GUIDED CASZ GENE EDITING TECHNOLOGY

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OTHER INFORMATION
KEYWORDS
CRISPR, gene editing, gene therapy, therapeutics,

CATEGORIZED AS
» Biotechnology
» Genomics
» Medical
» Gene Therapy
» Research Tools
» Therapeutics
» Research Tools
» Nucleic Acids/DNA/RNA
» Veterinary
» Other
» Therapeutics

RELATED CASES
2018-045-0
**PATENT STATUS**

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Additional Patents Pending

**BRIEF DESCRIPTION**

The CRISPR-Cas system is now understood to confer bacteria and archaea with acquired immunity against phage and viruses. CRISPR-Cas systems consist of Cas proteins, which are involved in acquisition, targeting and cleavage of foreign DNA or RNA, and a CRISPR array, which includes direct repeats flanking short spacer sequences that guide Cas proteins to their targets. Class 2 CRISPR-Cas systems are streamlined versions in which a single Cas protein bound to RNA is responsible for binding to and cleavage of a targeted sequence. The programmable nature of these minimal systems has facilitated their use as a versatile technology that is revolutionizing the field of genome manipulation, so there is a need in the art for additional Class 2 CRISPR/Cas systems (e.g., Cas protein plus guide RNA combinations).

UC Berkeley researchers discovered a new type of Cas protein, CasZ. (CasZ) is short compared to previously identified CRISPR-Cas endonucleases, and thus use of this protein as an alternative provides the advantage that the nucleotide sequence encoding the protein is relatively short. The researchers have shown that the CRISPR CasZ protein and its variants can be used in a complex for specific binding and cleavage of DNA. The CRISPR CasZ complex utilizes a novel RNA and a guide RNA to perform double stranded cleavage of DNA and the complex is expected to have a wide variety of applications in genome editing and nucleic acid manipulation.

**SUGGESTED USES**

- Genome editing
- Genetic engineering
- Gene therapy
- Research tools (e.g., high-throughput screening of gene functions in cell lines and in vivo)
- Creation of transgenic animal models
ADVANTAGES

» Adds additional versatility because of small size

» Variant PAM

ADDITIONAL TECHNOLOGIES BY THESE INVENTORS

▶ 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
▶ Type III CRISPR-Cas System for Robust RNA Knockdown and Imaging in Eukaryotes
▶ Cas12-mediated DNA Detection Reporter Molecules
▶ Improved guide RNA and Protein Design for CasX-based Gene Editing Platform
▶ Cas13a/C2c2 - A Dual Function Programmable RNA Endoribonuclease
▶ RNA-directed Cleavage and Modification of DNA using CasY (CRISPR-CasY)
▶ CasX Nickase Designs, Tans Cleavage Designs & Structure
▶ In Vivo Gene Editing Of Tau Locus Via Liponanoparticle Delivery
▶ CRISPR-CAS EFFECTOR POLYPEPTIDES AND METHODS OF USE THEREOF ("Cas-VariPhi")
▶ Modifications To Cas9 For Passive-Delivery Into Cells
▶ A Protein Inhibitor Of Cas9
▶ RNA-directed Cleavage and Modification of DNA using CasX (CRISPR-CasX)
▶ Compositions and Methods for Genome Editing
▶ Split-Cas9 For Regulatable Genome Engineering
▶ NANOPORE MEMBRANE DEVICE AND METHODS OF USE THEREOF
▶ Methods to Interfere with Prokaryotic and Phage Translation and Noncoding RNA
▶ CRISPR CASY COMPOSITIONS AND METHODS OF USE
▶ Single Conjugative Vector for Genome Editing by RNA-guided Transposition
▶ Improved Cas12a Proteins for Accurate and Efficient Genome Editing
▶ CRISPR-CAS EFFECTOR POLYPEPTIDES AND METHODS OF USE THEREOF
▶ Engineered/Variant Hyperactive CRISPR CasPhi Enzymes And Methods Of Use Thereof
▶ Engineering Cas12a Genome Editors with Minimized Trans-Activity
▶ 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)
▶ Structure-Guided Methods Of Cas9-Mediated Genome Engineering
▶ Endoribonucleases For Rna Detection And Analysis
▶ Efficient Site-Specific Integration Of New Genetic Information Into Human Cells
▶ CRISPR-Cas Effector Polypeptides and Methods of Use Thereof
▶ Class 2 CRISPR/Cas COMPOSITIONS AND METHODS OF USE
▶ Compositions and Methods of Use for Variant Csy4 Endoribonucleases
▶ Identification Of Sites For Internal Insertions Into Cas9
▶ Methods and Compositions for Controlling Gene Expression by RNA Processing