IMPROVED GUIDE RNA AND PROTEIN DESIGN FOR CASX-BASED GENE EDITING PLATFORM

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PATENT STATUS

Patent Pending

BRIEF DESCRIPTION

The inventors have developed two new CasX gene-editing platforms (DpbCasXv2 and PlmCasXv2) through rationale structural engineering of the CasX protein and gRNA, which yield improved in vitro and in vivo behaviors. These platforms dramatically increase DNA cleavage activity and can be used as the basis for further improving CasX tools.

The RNA-guided CRISPR-associated (Cas) protein CasX has been reported as a fundamentally distinct, RNA-guided platform compared to Cas9 and Cpf1. Structural studies revealed structural differences within the nucleotide-binding loops of CasX, with a compact protein size less than 1,000 amino acids, and guide RNA (gRNA) scaffold stem. These structural differences affect the active ternary complex assembly, leading to different in vivo and in vitro behaviors of these two enzymes.

SUGGESTED USES

Research and applications related to gene editing.

ADVANTAGES

Improved in vitro and in vivo behaviors of CasX and guide RNA.

RELATED MATERIALS


ADDITIONAL TECHNOLOGIES BY THESE INVENTORS

▶ Methods and Compositions for Using Argonaute to Modify a Single-Stranded Target Nucleic Acid
COMPOSITIONS AND METHODS FOR IDENTIFYING HOST CELL TARGET PROTEINS FOR TREATING RNA VIRUS INFECTIONS

- Lentivirus-like Particle Delivery of CRISPR-Cas9 & Guide RNA for Gene Editing
- Cas9 Variants With Altered DNA Cleaving Activity
- Cas12-mediated DNA Detection Reporter Molecules
- Cas13a/C2c2 - A Dual Function Programmable RNA Endoribonuclease
- Methods For High Signal-To-Noise Imaging Of Chromosomal Loci In Cells Using Fluorescent Cas9
- A Dual-RNA Guided Cas2 Gene Editing Technology
- CRISPR-CAS EFFECTOR POLYPEPTIDES AND METHODS OF USE THEREOF ("Cas-VarPhi")
- A Protein Inhibitor Of Cas9
- Small Cas9 Protein Inhibitor
- Split-Cas9 For Regulatable Genome Engineering
- Optimized Virus-like Particles for Cas9 RNPs & Transgene/HDR Template Delivery
- CRISPR-CAS EFFECTOR POLYPEPTIDES AND METHODS OF USE THEREOF ("Cas-Theta")
- Protein Inhibitor of Type VI-B CRISPR-Cas System
- COMPOSITIONS AND METHODS FOR INCREASING HOMOLOGY-DIRECTED REPAIR
- 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 ("Cas-Omega")
- Protein Inhibitor of Type II-A CRISPR-Cas System
- CRISPR-CAS EFFECTOR POLYPEPTIDES AND METHODS OF USE THEREOF
  - Engineered/Variant Hyperactive CRISPR CasPhi Enzymes And Methods Of Use Thereof
- 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 (CasGamma)
- 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
- Chimeric Cas9 Variants With Novel Engineered Enzymatic Activities
- Small Molecule Assisted Cell Penetrating Cas9 RNP Delivery
- Methods and Compositions for Controlling Gene Expression by RNA Processing