

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

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## PATENT STATUS

Country	Type	Number	Dated	Case
United States Of America	Published Application	20230407276	12/21/2023	2021-064
European Patent Office	Published Application	4256045 A0	10/11/2023	2021-064

## 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

## CONTACT

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## INVENTORS

» Doudna, Jennifer A.

## OTHER INFORMATION

### CATEGORIZED AS

» **Agriculture & Animal Science**

» Animal Science

» Other

» Transgenics

» **Biotechnology**

» Genomics

» **Energy**

» Bioenergy

» **Engineering**

» Engineering

» **Imaging**

» Medical

» Molecular

» **Medical**

» Gene Therapy

» Imaging

» Research Tools

» Therapeutics

» **Research Tools**

» Other

## RELATED CASES

2021-064-0

## 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
- ▶ Tissue-Specific Genome Engineering Using CRISPR-Cas9
- ▶ Type III CRISPR-Cas System for Robust RNA Knockdown and Imaging in Eukaryotes
- ▶ Cas12-mediated DNA Detection Reporter Molecules
- ▶ 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)
- ▶ CasX Nickase Designs, Tans Cleavage Designs & Structure
- ▶ In Vivo Gene Editing Of Tau Locus Via Liponanoparticle Delivery
- ▶ Methods and Compositions for Modifying a single stranded Target Nucleic Acid
- ▶ A Dual-RNA Guided CasZ Gene Editing Technology
- ▶ CRISPR-CAS EFFECTOR POLYPEPTIDES AND METHODS OF USE THEREOF ("Cas-VariPhi")
- ▶ A Protein Inhibitor Of Cas9
- ▶ RNA-directed Cleavage and Modification of DNA using CasX (CRISPR-CasX)
- ▶ Compositions and Methods for Genome Editing
- ▶ Methods to Interfere with Prokaryotic and Phage Translation and Noncoding RNA
- ▶ 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
- ▶ 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
- ▶ 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
- ▶ Structure-Guided Methods Of Cas9-Mediated Genome Engineering
- ▶ 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
- ▶ Methods and Compositions for Controlling Gene Expression by RNA Processing