

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

Tech ID: 32199 / UC Case 2021-064-0

## PATENT STATUS

Country	Type	Number	Dated	Case
United States Of America	Published Application	<a href="#">20230407276</a>	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

Terri Sale  
[terri.sale@berkeley.edu](mailto:terri.sale@berkeley.edu)  
tel: 510-643-4219.



## 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
- ▶ 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
- ▶ 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
- ▶ A Dual-RNA Guided CasZ Gene Editing Technology
- ▶ 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
- ▶ 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



### University of California, Berkeley Office of Technology Licensing

2150 Shattuck Avenue, Suite 510, Berkeley, CA 94704

Tel: 510.643.7201 | Fax: 510.642.4566

[ipira.berkeley.edu/](http://ipira.berkeley.edu/) | [otl-feedback@lists.berkeley.edu](mailto:otl-feedback@lists.berkeley.edu)

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