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## EFFICIENT SITE-SPECIFIC INTEGRATION OF NEW GENETIC INFORMATION INTO HUMAN CELLS

Tech ID: 24215 / UC Case 2014-199-0

### PATENT STATUS

Country	Type	Number	Dated	Case
United States Of America	Issued Patent	10,570,418	02/25/2020	2014-199

### BRIEF DESCRIPTION

The CRISPR/Cas9 system is a robust genome editing technology that works based on the RNA-programmed DNA cleaving activity of the Cas9 enzyme. Cas9 generates blunt double-strand DNA (dsDNA) breaks (DSBs) at sites defined by a guide sequence contained within an associated CRISPR RNA (crRNA) transcript and has transformed the ability to engineer eukaryotic organisms by initiating DNA repair pathways that lead to targeted genetic re-programming. There is a need, however, for methods that increase the efficiency of target DNA modification by Cas9 targeting complexes and/or increase the effectiveness of such methods.

UC Berkeley researchers have found a simple and robust approach for introducing new genetic information site-specifically, in a eukaryotic cell, and with high efficiency using timed delivery of Cas9-guide RNA ribonucleoprotein (RNP) complexes. The approach combines well-established cell cycle synchronization techniques with direct nucleofection of the pre-assembled Cas9 ribonucleoprotein (RNP) complexes to achieve controlled nuclease action at the phase of the cell cycle best for HDR.

### SUGGESTED USES

- » Genome editing (e.g., human, plants, and animal cells)
- » Genome engineering in both transformed and primary human cells

### ADVANTAGES

- » Enhanced HDR efficiency in human cells
- » Highly efficient, site-specific editing
- » Simple and robust editing
- » Cell mortality minimized

### PUBLICATION

### CONTACT

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### INVENTORS

- » Doudna, Jennifer A.

### OTHER INFORMATION

#### KEYWORDS

Genetic engineering, homology-directed repair, HDR, genome editing, Cas9, CRISPR

#### CATEGORIZED AS

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

#### RELATED CASES

2014-199-0

## 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
- ▶ 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
- ▶ 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
- ▶ Methods and Compositions for Modifying a single stranded Target Nucleic Acid
- ▶ 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
- ▶ Improved Cas12a Proteins for Accurate and Efficient Genome Editing
- ▶ CRISPR-CAS EFFECTOR POLYPEPTIDES 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
- ▶ 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