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IN VITRO AND IN VIVO GENOME EDITING BY LNP DELIVERY OF CRISPR RIBONUCLEOPROTEIN

Tech ID: 33667 / UC Case 2025-005-0

PATENT STATUS

Patent Pending

BRIEF DESCRIPTION

Although viral delivery of CRISPR genome editors is the most widely used method for in vivo cell editing, viral vectors can be immunogenic, carry the risk of vector genome integration and can induce off-target DNA damage due to continuous genome editor expression. Lipid-nanoparticle (LNP):mRNA complexes are non-virally derived vehicles for in vivo delivery that have provided for genome editing in the liver. However, developing LNP:mRNA complexes that can edit non-liver tissues remains a challenge.

UCB researchers have created new LNP compositions and methods for delivery that have increased efficiency for delivering a molecular payload such as CRISPR-Cas effector proteins, guide RNAs, and/ nucleic acids encoding same.

SUGGESTED USES

» gene delivery

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INVENTORS

» Doudna, Jennifer A.

OTHER INFORMATION

CATEGORIZED AS

- » Medical
 - » Delivery Systems
 - >> Therapeutics
- » Research Tools
 - » Nucleic Acids/DNA/RNA

RELATED CASES2025-005-0

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
- ▶ Compositions and Methods for Delivering Molecular Cargo to Cells
- 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
- ▶ IS110 and IS1111 Family RNA-Guided Transposons
- Methods to Interfere with Prokaryotic and Phage Translation and Noncoding RNA
- ▶ Variant Cas12a Protein Compositions and Methods of Use
- ► 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
- ▶ 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
- ► Immune Cell-Mediated Intercellular Delivery Of Biomolecules
- Methods and Compositions for Controlling Gene Expression by RNA Processing



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