TISSUE-SPECIFIC GENOME ENGINEERING USING CRISPR-CAS9

Tech ID: 25143 / UC Case 2015-205-0

PATENT STATUS

Country	Type	Number	Dated	Case
Japan	Issued Patent	7268103	04/24/2023	2015-205
United States Of America	Issued Patent	10,851,367	12/01/2020	2015-205
Japan	Published Application	P2018-537448A	12/20/2018	2015-205
European Patent Office	Published Application	3 373 979	09/19/2018	2015-205
Argentina	Published Application	AR 106639 A1	02/07/2018	2015-205

Additional Patents Pending

BRIEF DESCRIPTION

Delivering gene-editing agents safely and effectively has long been a challenge in modern medicine. Traditional methods using viral vectors introduce risks such as insertional mutagenesis, hepatotoxicity, and transient therapeutic effects due to immune responses. UC Berkeley researchers have developed a groundbreaking tissue-specific genome engineering system utilizing CRISPR-Cas9, offering a safer and more precise alternative for gene therapy.

UC Berkeley researchers and others have created compounds, compositions, uses thereof the combines the cutting-edge CRISPR technology with advanced targeting mechanishms for the treatment of diseases, conditions and/or disorders, and uses thereof as asialoglycoprotein receptor (ASGPR) targeting agents.

SUGGESTED USES

» Gene editing

» Gene delivery

ADVANTAGES

» By leveraging ASGPR targeting agents, this approach ensures precise uptake by hepatocytes and other specific tissues.

» Avoids the risks associated with viral vectors, reducing toxicity and immune complications.

» By delivering gene-editing agents in protein form rather than DNA or RNA, the technology maximizes therapeutic potential while minimizing unwanted genetic disruptions.

» Overcomes the limitations of receptor-mediated endocytosis to ensure the therapeutic agents effectively reach their intended subcellular locations.

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
- Cas9 Variants With Altered DNA Cleaving Activity

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OTHER INFORMATION

CATEGORIZED AS

» **Medical**

» Gene Therapy

» Research Tools

» Therapeutics

RELATED CASES

2015-205-0

- 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
- 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
- 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
- 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

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