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IN VIVO GENE EDITING OF TAU LOCUS VIA LIPONANOPARTICLE DELIVERY

Tech ID: 33310 / UC Case 2024-026-0

PATENT STATUS

Patent Pending

BRIEF DESCRIPTION

Delivery technologies such as lipid nanoparticles (LNP) offer significant advantages over the delivery of free RNA for various RNA therapeutic, vaccine, and basic science applications.

UC Berkeley researchers developed a new class of lipid nanoparticle (LNP) which is effective in delivering various types of nuclei acids in different tissues. The LNP was successfully tested in in-vivo mouse models and therefore poses a significant promise in the gene editing field. The lipid formulation was packaged together with CRISPR Cas9 and a gRNA targeting the endogenous Tau locus. Tau dysrregulation is a pathological feature of Alzheimers disease, thus the invention provides a means to intervene in the development of pathological states associated with Tau aggregate formation.

SUGGESTED USES

» therapeutic applications, particularly ones targeting the Tau locus (e.g., Alzheimers disease)

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INVENTORS

» Doudna, Jennifer A.

OTHER INFORMATION

CATEGORIZED AS

- » Biotechnology
 - » Genomics
- » Medical
 - » Disease: Central Nervous

System

» Therapeutics

RELATED CASES

2024-026-0

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
- ▶ Improved guide RNA and Protein Design for CasX-based Gene Editing Platform
- 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
- ▶ 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



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