

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 dysrrregulation 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)

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
- ▶ Highly Multiplexed Tagging Methods for RNA Imaging and Other Applications
- ▶ 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
- ▶ 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)

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INVENTORS

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OTHER INFORMATION

CATEGORIZED AS

- » **Biotechnology**
- » Genomics
- » **Medical**
- » Disease: Central Nervous System
- » Therapeutics

RELATED CASES

2024-026-0

- ▶ Compositions and Methods for Genome Editing
- ▶ Methods to Interfere with Prokaryotic and Phage Translation and Noncoding RNA
- ▶ Minimal RNA Targeting CRISPR Cas Systems
- ▶ 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)
- ▶ Structure-Guided Methods Of Cas9-Mediated Genome Engineering
- ▶ Efficient Site-Specific Integration Of New Genetic Information Into Human Cells
- ▶ Virus-encoded DNA-binding Proteins
- ▶ 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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