COMPOSITIONS AND METHODS FOR DELIVERING MOLECULAR CARGO TO CELLS

Tech ID: 33916 / UC Case 2025-088-0

PATENT STATUS

Patent Pending

BRIEF DESCRIPTION

Efficient delivery and expression of exogenous proteins in cell populations (e.g., cells in the body) for gene therapy / gene editing applications, is an important goal in biomedicine. This can be hampered by inefficient transport of enzymes from outside the body to cells within the body. When delivering nucleic acids or proteins of interest (e.g., DNA editing enzymes), most delivery methods can only reach and enter a small subset of cells within a tissue. There is a need for compositions and methods for improved delivery of proteins of interest, and such is provided herein.

UC Berkeley researchers have discovered that delivery of a molecular cargo to a target cell can be more efficiently achieved by using a cell as the delivery vehicle. This can be accomplished by delivering a nucleic acid encoding an enveloped delivery vehicle (EDV) (one that comprises a molecular cargo), to a producer cell where the producer cell produces the EDV and thereby delivers the molecular cargo to neighboring cells (referred to herein as receiver cells). Thus, there is no human intervention between delivery of a subject nucleic acid (encoding the EDV) and subsequent delivery of EDVs to target cells (receiver cells).

SUGGESTED USES

» Direct in vivo delivery of CRISPR-Cas genome editing machinery to treat genetic disorders such as Duchenne muscular dystrophy

» Targeted gene therapy for tissues with typically low transfection efficiency, such as the liver or muscle

» Local secretion and distribution of therapeutic proteins or antibodies directly at a disease site.

» Research tools for studying cell-to-cell molecular transport and localized cargo propagation

ADVANTAGES

» Utilizes host cells to produce and spread delivery vehicles, extending the therapeutic reach beyond the primary injection site

» Removes the need for complex and costly ex vivo production and purification of delivery particles

» Achieves broad tissue-level editing even with low initial transfection rates

» Integrates easily with existing delivery platforms, including lipid nanoparticles and viral vectors

» Employs a minimal structural protein that streamlines assembly and can be programmed for specific cell tropism

ADDITIONAL TECHNOLOGIES BY THESE INVENTORS

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INVENTORS

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OTHER INFORMATION

CATEGORIZED AS

» **Biotechnology**
» Genomics
» **Medical**
» Gene Therapy
» Therapeutics
» Vaccines
» **Research Tools**
» Nucleic Acids/DNA/RNA
» **Veterinary**
» Therapeutics
» Vaccines

RELATED CASES

2025-088-0

- 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)
- Generation of Chimeric RNA with Type III CRISPR-Cas
- 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
- 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
- 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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