OPTIMIZED VIRUS-LIKE PARTICLES FOR CAS9 RNPS & TRANSGENE/HDR TEMPLATE DELIVERY

Tech ID: 32400 / UC Case 2021-177-0

PATENT STATUS

Patent Pending

BRIEF DESCRIPTION

The inventors have developed optimized methods for using virus-like particles for the co-delivery of Cas9 ribonucleoprotein complexes and:

» a lentiviral genome that encodes a large transgene, such as a chimeric antigen receptor (CAR) transgene
» a lentiviral genome that does not encode a sgRNA expression cassette
» a method for nucleofecting VLPs + homology directed repair (HDR) donor template together to enhance HDR in treated cells

SUGGESTED USES

In vivo/ex vivo generation of CRISPR-Cas9 gene edited CAR T cells.

Any application where a simultaneous gene knockout and transgene introduction/expression is desired.

ADVANTAGES

This technology offers a one-step strategy using engineered lentiviral particles to introduce Cas9 RNPs and a CAR transgene into primary human T cells without electroporation. Furthermore, programming particle tropism allowed the inventors to target a specific cell type within a mixed cell population. This adaptable approach to immune cell engineering ex vivo provides a strategy applicable to genetic modification of targeted somatic cells in vivo.

RELATED MATERIALS

Suggested Uses

In vivo/ex vivo generation of CRISPR-Cas9 gene edited CAR T cells.

Any application where a simultaneous gene knockout and transgene introduction/expression is desired.

ADDITIONAL TECHNOLOGIES BY THESE INVENTORS

» Methods and Compositions for Using Argonaute to Modify a Single-Stranded Target Nucleic Acid
» COMPOSITIONS AND METHODS FOR IDENTIFYING HOST CELL TARGET PROTEINS FOR TREATING RNA VIRUS INFECTIONS
» 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 Endonuclease
» Methods For High Signal-To-Noise Imaging Of Chromosomal Loci In Cells Using Fluorescent Cas9
» A Dual-RNA Guided Cas2 Gene Editing Technology
» MODULATORS OF TYPE VI-D CRISPR-CAS EFFECTOR POLYPEPTIDES AND METHODS OF USE THEREOF
» CRISPR-CAS EFFECTOR POLYPEPTIDES AND METHODS OF USE THEREOF ("Cas-VarPhi")
» A Protein Inhibitor Of Cas9
» Small Cas9 Protein Inhibitor
» Split-Cas9 For Regulatable Genome Engineering
» Decorating Chromatin for Precise Genome Editing Using CRISPR
» CRISPR-CAS EFFECTOR POLYPEPTIDES AND METHODS OF USE THEREOF ("Cas-Theta")
» COMPOSITIONS AND METHODS FOR INCREASING HOMOLOGY-DIRECTED REPAIR
» CRISPR CASY COMPOSITIONS AND METHODS OF USE
» Single Conjugative Vector for Genome Editing by RNA-guided Transposition
» CRISPR-CAS EFFECTOR POLYPEPTIDES AND METHODS OF USE THEREOF ("Cas-Omega")
» CRISPR-CAS EFFECTOR POLYPEPTIDES AND METHODS OF USE THEREOF
» Engineered/Variant Hyperactive CRISPR Cas9i Enzymes And Methods Of Use Thereof
» Type V CRISPR/CAS Effectors for Cleaving ssDNA and Detecting Target DNA

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- 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 (CasGamma)
- Class 2 CRISPR/Cas Compositions and Methods of Use
- Compositions and Methods of Use for Variant Csy4 Endonucleases
- Identification Of Sites For Internal Insertions Into Cas9
- Chimeric Cas9 Variants With Novel Engineered Enzymatic Activities
- Small Molecule Assisted Cell Penetrating Cas9 RNP Delivery
- Methods and Compositions for Controlling Gene Expression by RNA Processing