SMALL MOLECULE ASSISTED CELL PENETRATING CAS9 RNP DELIVERY

Tech ID: 27354 / UC Case 2017-098-0

PATENT STATUS

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<tr>
<td>United States Of America</td>
<td>Published Application</td>
<td>20200115688</td>
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<td>Patent Cooperation Treaty</td>
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<td>WO2019036185</td>
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Additional Patent Pending

BRIEF DESCRIPTION

Clustered regularly interspaced short palindromic repeats (CRISPR) Cas systems provide a means for modifying genomic information and have the potential to revolutionize the treatment of genetic diseases. Although RNA-programmed Cas9 has proven to be a versatile tool for genome engineering in multiple cell types and organisms, it has been challenging to develop the therapeutics because they require the simultaneous in vivo delivery of the Cas9 protein, guide RNA and donor DNA. Compositions that can increase the efficiency of such delivery, particular in eukaryotic cells, are greatly needed.

UC Researchers have discovered that the inclusion of an agent that decreases the acidity of an endosome inside eukaryotic cells, in a genome editing composition, increased the efficiency of genome editing. The agent was included in a composition having an RNA-guided endonuclease and an RNA-guided endonuclease and was used for gene editing.

SUGGESTED USES

- Genome editing (particularly in eukaryotic cells)
- Research reagent
- Gene therapy
- Increased Delivery of CRISPR Cas components to eukaryotic cells

ADVANTAGES

- Significantly increased gene editing efficiency through increased delivery through endosome

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
- 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 CasZ Gene Editing Technology
- 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 CAS9 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 ("Cas-Omega")
- CRISPR-CAS EFFECTOR POLYPEPTIDES AND METHODS OF USE THEREOF
- 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 (CasGamma)
- Improved gRNA and Protein Design for CasX-based Gene Editing Platform
- 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
- Chimeric Cas9 Variants With Novel Engineered Enzymatic Activities
- Methods and Compositions for Controlling Gene Expression by RNA Processing