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>
<td>04/16/2020</td>
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<td>Patent Cooperation Treaty</td>
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<td>WO2019036185</td>
<td>02/21/2019</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

INVENTORS

» Doudna, Jennifer A.

OTHER INFORMATION

KEYWORDS

CRISPR, delivery, eukaryotic cells

CATEGORIZED AS

» Medical
» Delivery Systems
» Gene Therapy
» Research Tools
» Research Tools
» Nucleic Acids/DNA/RNA

RELATED CASES

2017-098-0

ADDITIONAL TECHNOLOGIES BY THESE INVENTORS

- COMPOSITIONS AND METHODS FOR IDENTIFYING HOST CELL TARGET PROTEINS FOR TREATING RNA VIRUS INFECTIONS
- Lentivirus-like Particle Delivery of CRISPR-Cas9 & Guide RNA for Gene Editing
- 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 Endonuclease
- CasX Nickase Designs, Tans Cleavage Designs & Structure
- A Dual-RNA Guided Cas2 Gene Editing Technology
- CRISPR-CAS EFFECTOR POLYPEPTIDES AND METHODS OF USE THEREOF ("Cas-VarPh")
- Modifications To Cas9 For Passive-Delivery Into Cells
- A Protein Inhibitor Of Cas9
- Split-Cas9 For Regulatable Genome Engineering
- NANOPORE MEMBRANE DEVICE AND METHODS OF USE THEREOF
- Optimized Virus-like Particles for Cas9 RNPs & Transgene/HDR Template Delivery
- Protein Inhibitor of Type VI-B CRISPR-Cas System
- 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