Peptide Mediated Oligonucleotide Delivery to the CNS

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BACKGROUND

Many diseases of the central nervous system (CNS) arise from the accumulation of proteins such as α-synuclein (aSyn) in Parkinson's Disease (PD) or Aß in Alzheimer's disease (AD). The ability to regulate the expression at the gene transcription level would be beneficial for reducing the accumulation of these proteins or regulating expression levels of other genes in the CNS. aSyn also accumulates in other neurodegenerative diseases including dementia with Lewy Body (DLB), multiple system atrophy (MSA) and Gaucher's disease. This means that regulation of aSyn expression may be crucial to the therapeutic control of numerous neurodegenerative diseases.

TECHNOLOGY DESCRIPTION

Researchers at UC San Diego have developed a novel peptide ($ApoB^{11}$) and method for the delivery of anti-sense oligonucleotides (ASO) to the CNS by intra-venous delivery. The $ApoB^{11}$ peptide is derived from the apolipoprotein B protein with the addition of a glycine linker and arginine tail. This peptide allows the delivery of ASOs to the brain following the less-invasive intra-venous or even intra-peritoneal injection versus the current intra-thecal injection route for CNS ASO delivery used now.

APPLICATIONS

The $ApoB^{11}$ peptide could be used to deliver any form or design of nucleotides including, but not limited to: cDNA, mRNA, siRNA, shRNA, miRNA, gRNA or synthetic nucleotide sequences. Delivery of oligonucleotides to the CNS could be beneficial for neurodegenerative diseases, neuro-modulatory treatments, cancers of the CNS, gene targeted editing of the CNS or anti-microbial gene delivery of the CNS.

ADVANTAGES

The peptide utilized in this invention is an endogenous peptide that would be less likely to elicit an immune response than a foreign, virus-based peptide. Additionally, we have ample evidence showing that delivery with this peptide is possible to a wide variety of cells in the CNS.

STATE OF DEVELOPMENT

The project is complete and has been shown to work in an animal model of disease.

INTELLECTUAL PROPERTY INFO

The technology is patent pending and is available for licensing.

RELATED MATERIALS

▶ Spencer B, Trinh I, Rockenstein E, Mante M, Florio J, Adame A, El-Agnaf OMA, Kim C, Masliah E, Rissman RA. Systemic peptide mediated delivery of an siRNA targeting a-syn in the CNS ameliorates the neurodegenerative process in a transgenic model of Lewy body disease. Neurobiol Dis. 2019 Jul;127:163-177. doi: 10.1016/j.nbd.2019.03.001. Epub 2019 Mar 5. - 03/05/2019

PATENT STATUS

Patent Pending

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OTHER INFORMATION

KEYWORDS

a-synuclein, Parkinson's Disease, Alzheimer's disease, CNS, peptide delivery,

CATEGORIZED AS

- **Medical**
  - Delivery Systems
  - Disease: Central Nervous System

RELATED CASES

2019-038-0