HUMAN CENTRAL NERVOUS SYSTEM (CNS) TARGETING AAV VARIANTS

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PATENT STATUS

Patent Pending

BRIEF DESCRIPTION

Researchers at UCSF and UC Berkeley have developed a recombinant adeno-associated virus (rAAV) with an altered capsid protein, where the rAAV exhibits greater ability to infect a central nervous system cell compared to wild-type AAVs.

The central nervous system (CNS) comprises a multitude of cell types with diverse functionality and specialization. Dysregulation of neuronal or glial (including microglial) populations has been implicated in multiple disorders, including Alzheimer's, Parkinson's, Multiple Sclerosis and Huntington's disease. AAVs hold tremendous promise as a gene delivery vector to treat such conditions given their reasonable starting efficiency and safety profile. However, challenges in efficient and targeted delivery to specific cell populations make strategies employing these vectors in the CNS particularly challenging.

Stage of Research

The inventors have developed a recombinant AAV with an altered capsid protein, where the rAAV exhibits greater ability to infect a CNS cell compared to wild-type AAV.

SUGGESTED USES

Glial cell infection using evolved AAV variants across multiple regions of primary brain tissue

Transduction towards microglia cells using evolved AAV variants

Expression of AAV-GFP transduced in human adult brain tissue in microglial cells

AAV-GFP transduction in various glial cell types in prenatal human brain tissue

ADVANTAGES

AAV virions are composed of a 25 nm icosahedral capsid that mediates the ability of AAV vectors to overcome many of the biological barriers of viral transduction, including cell surface receptor binding, endocytosis, intracellular trafficking, and unpacking in the nucleus.

RELATED MATERIALS

ADDITIONAL TECHNOLOGIES BY THESE INVENTORS

» 3D System For Differentiation Of Oligodendocyte Precusors From Pluripotent Stem Cells

» Membrane-Associated Accessory Protein Variants Confer Increased AAV Production

» Self-Inactivating Targeted DNA Nucleases For Gene Therapy

» Improving Packaging and Diversity of AAV Libraries with Machine Learning

INVENTORS

» Schaffer, David V.

OTHER INFORMATION

KEYWORDS

Adeno-associated virus, neuron, brain

CATEGORIZED AS

» **Medical**

   » Delivery Systems

RELATED CASES

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