

Modified SYNGAP1 Protein Expressed in a Lentiviral Vector for the Treatment of Patients with SYNGAP1-related Intellectual Disability

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ABSTRACT

Researchers at the University of California, Davis have developed a novel stem cell gene therapy approach utilizing a modified SYNGAP1 protein to treat Synaptic Ras GTPase Activating Protein 1-related intellectual disability (SRID).

FULL DESCRIPTION

This technology involves the modification of the wild type SYNGAP1 protein to include a secretion signal and additional N-glycan sites, allowing for its secretion and uptake by neurons. The modified SYNGAP1 gene is cloned into a lentiviral vector for expression in targeted cells, specifically designed for transduction of human CD34+ hematopoietic stem and progenitor cells. This approach aims to deliver therapeutic levels of functional SYNGAP1 to affected neurons, offering a potential treatment strategy for SRID and other neurodevelopmental disorders.

APPLICATIONS

- ▶ Gene therapy for Synaptic Ras GTPase Activating Protein 1-related intellectual disability (SRID) and other neurodevelopmental disorders.
- ▶ Stem cell therapies for genetic and neurodegenerative diseases.
- ▶ Biotechnological research and development in gene modification and delivery systems.

FEATURES/BENEFITS

- ▶ Introduction of a secretion signal and N-glycan sites enhances the delivery and uptake of SynGAP1 by neurons.
- ▶ Utilizes lentiviral vectors for stable transduction and expression in target cells.
- ▶ Enables cross-correction by allowing gene-modified hematopoietic stem cells to differentiate into microglia and deliver functional SynGAP1.
- ▶ Has shown significant improvement in SRID-related phenotypes in a mouse model.

PATENT STATUS

Patent Pending

RELATED MATERIALS

► Hematopoietic stem cell gene therapy for the treatment of SYNGAP1-related non-specific intellectual disability - 07/05/2024

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OTHER INFORMATION

KEYWORDS

SYNGAP1, lentiviral
vector, hematopoietic
stem cells, gene therapy,
neurodevelopmental
disorders

CATEGORIZED AS

Medical

▶ Disease: Central Nervous System

- ▶ Stem Cell
- ► Therapeutics

RELATED CASES

2024-501-0

ADDITIONAL TECHNOLOGIES BY THESE INVENTORS

► HIV Gene Therapy Treatment

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