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

Tech ID: 34330 / UC Case 2024-501-0

CONTACT

Raj Gururajan
rgururajan@ucdavis.edu
tel: 530-754-7637.

INVENTORS

- Anderson, Joseph

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

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

Country	Type	Number	Dated	Case
Patent Cooperation Treaty	Published Application	2025255491	12/11/2025	2024-501

Additional Patent Pending

RELATED MATERIALS

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

ADDITIONAL TECHNOLOGIES BY THESE INVENTORS

- HIV Gene Therapy Treatment

University of California, Davis
Technology Transfer Office
1 Shields Avenue, Mrak Hall 4th Floor,
Tel: 530.754.8649
techtransfer@ucdavis.edu

Davis,CA 95616

https://research.ucdavis.edu/technology-transfer/

Fax:

530.754.7620