Cincinnati Children's | Innovation Ventures

Metabolic disorder

Autologous cell and gene therapy for Gaucher Disease

Brief Description of Technology

Therapy for neuronopathic Gaucher Disease using gene therapy and a specialized neuronal lineage iPS cell that facilitates entry to and engraftment in the brain.

TECHNOLOGY ID

2016-1202

BUSINESS OPPORTUNITY

Exclusive License

TECHNOLOGY TYPE

Biologic Therapy

PATENT INFORMATION

US Non-Provisional Filing

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Technology Overview

Cincinnati Children's Hospital researchers in the Department of Human Genetics have developed a therapeutic approach to treat neuronopathic Gaucher Disease. Gaucher Disease is a rare pediatric lysosomal storage disorder resulting from mutations in the acid beta-glucocerebrosidase gene, with a frequency of about 1 in 57,000 live births. Over 400 mutations have been identified within the GCase coding gene, GBA1, resulting in heterogeneous disease phenotypes. Insufficient GCase activity leads to progressive accumulation of its substrates, glucosylceramide (GC) and glucosylsphingosine (GS), resulting in a continuum of clinical phenotypes in visceral organs and the central nervous system (CNS). Dr. Ying Sun's research group has developed a therapeutic approach to treatment of the neuronopathic form of Gaucher Disease using autologous neuronal precursor cells that are selected for high expression of Very Late Antigen-4. These cells are then enhanced with a lentiviral construct encoding GCase. Non-invasive IV administration in an animal model of disease demonstrated engraftment in the brain (due to VCAM1 mediated BBB transport) and increased GCase activity, with prolonged survival and improved sensorimotor function.

Applications

This technology is a non-invasive iPSC-based cell therapy for Gaucher Disease. This novel therapeutic approach allows cell treatment to be administered into the peripheral bloodstream where they can then make their way to the brain through naturally existing uptake pathways. This approach is superior to existing approaches which require delivering cells directly into the brain or CNS, which is highly invasive and risky to the patient.

Advantages

*Specialized subset of NP cells allows brain targeting and engraftment *Non or low invasive administration *Autologous cell therapy approach combined with well-characterized lentiviral GT

Market Overview

Gaucher Disease is an autosomal recessive disorder resulting from

defective function of acid beta-glucocerebrosidase in the lysosome. It is a common lysosomal storage disease with a frequency of about 1 in 57,000 live births with a slightly higher prevalence in people of Ashkenazi Jewish Ancestry.

Investigator Overview

Dr. Ying Sun, Professor, Division of Human Genetics