BASAL FOREBRAIN CHOLINERGIC NEURONS

Mount Sinai

NYSCF The New York Stem Cell Foundation Research Institute

CELL-BASED THERAPEUTIC STRATEGY FOR ALZHEIMER'S DISEASE

METHOD AND COMPOSITION FOR GENERATING BASAL FOREBRAIN CHOLINERGIC NEURONS (BFCNs)

PATENT PENDING

Australia (AU)
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15/990,208 (U.S.)
WO/2018/218193 (WO)

MOUNT SINAI / NYSCF PAPER

CRISPR/Cas9-Correctable mutation-related molecular and physiological phenotypes in iPSC-derived Alzheimer's PSEN2^{N141I} neurons.

Acta Neuropathologica Communications. 2017.

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Overview

Alzheimer's disease (AD) is a progressive disease resulting in senile dementia. There are 50 million people affected by dementia worldwide and, according to Alzheimer's Disease International, this number will grow to 152 million by 2050. The annual cost is an estimated $1 trillion and growing.

There are currently no disease-modifying treatments available to patients, but studies have revealed that human embryonic stem cell-derived basal forebrain cholinergic neurons (BFCNs) transplanted into AD mouse models can be associated with cognitive improvement in the affected mice. These findings highlight the relevance and potential strategy for cell-based therapeutic treatments moving forward. However, to move forward, a refined differentiation protocol to generate human BFCNs is needed.

Technology Summary

BFCNs are one of the most vulnerable neuronal populations associated with cognitive decline in AD patients. Pluripotent stem cell (PSC) generated BFCNs demonstrate a potential strategy for subtype-specific cell-based therapies to treat AD. The present invention provides various new and improved methods for the generation of BFCNs from PSCs, including embryonic and induced pluripotent stem cells. These methods are highly reproducible, efficiently deriving BFCNs across various PSCs.

Inventor Profiles

Dr. Sam Gandy, M.D., Ph.D., is Mount Sinai Professor of Alzheimer's Disease Research, Professor of Neurology and Psychiatry, Associate Director of the Mount Sinai Alzheimer's Disease Research Center in New York City, and Chairman Emeritus of the National Medical and Scientific Advisory Council of the Alzheimer's Association. Dr. Gandy is an international expert in the metabolism of the sticky substance called amyloid that clogs the brain in patients with AD. Dr. Gandy has written more than 250 original papers, chapters, and reviews on this topic. Dr. Gandy has received continuous NIH funding for his research on amyloid metabolism since 1986.

Dr. Scott Noggle, Ph.D., is the Senior VP of Research at the NYSCF Research Institute and oversees all stem cell research programs. Dr. Noggle applies new advances in PSC biology and cell reprogramming to the creation of human models of neurodegenerative diseases, with a focus on AD, to discover new disease targets. He also directs a group at the NYSCF Research Institute developing large-scale automated systems that use stem cells as a tool to understand how genetics impact susceptibility to these diseases. He and his team have developed high throughput automated systems for deriving new stem cell lines and differentiated cells to study disease models from large numbers of patients in parallel.

Dr. Maitane Ortiz-Virumbrales, Ph.D., Dr. Ilya Kruglikov, Ph.D., and Dr. Michelle Ehrlich, M.D., are co-inventors.