NYSCF INDUCED NEURONS

NYSCF The New York Stem Cell Foundation Research Institute

FUNCTIONAL NEURONS CONVERTED DIRECTLY FROM FIBROBLASTS

GENE EXPRESSION SYSTEM FOR INDUCED NEURONS

PATENT PENDING

Australia (AU); Brazil (BR); Canada (CA); China (CN); Europe (EP); Hong Kong (HK); Israel (IL); India (IN); Japan (JP); Mexico (MX); New Zealand (NZ); Russia (RU); South Korea (KR); United States (U.S.)

LEARN MORE

WO/2018/206798 (WO)

READ MORE

Dual Modulation of Neuron-Specific microRNAs and the REST Complex Promotes Functional Maturation of Huan Adult Induced Neurons.

Birtele M, Sharma Y, Kidnapillai S, Lau S, Stoker T, Barker R, Ottosson D, Drouin-Ouellet J, Parmar M. FEBS. 2019.

CONTACT US

partnering@NYSCF.org

Overview

New advances in somatic cell reprogramming offer unique access to human neurons from defined patient groups for modeling neurological disorders *in vitro*. This has enabled a number of mechanistic studies to better understand how pathology arises and develops, and also creates new opportunities for early and differential diagnostic tests and drug screens.

As an alternative for generating disease and patient specific neurons, adult fibroblasts can be directly converted into functional neurons using chemicals, defined sets of transcription factors or microRNAs (miRNAs) for chemical reprogramming. This type of direct reprograming allows fibroblasts to be converted into induced neurons (iNs) without transitioning via a proliferative stem cell intermediate, making the process faster and easier. In addition, recent studies have demonstrated that the resulting iNs, unlike induced pluripotent stem cells (iPSCs), maintain the ageing signature of the donor, making iNs ideal candidates for modeling neuronal pathology in late-onset diseases. However, a number of factors limits the reprogramming efficiency of this approach.

Technology Summary

The present invention relates to gene expression systems for use in obtaining iNs from adult fibroblast cells. The invention is an all-in-one neural conversion vector that contains all the components necessary for robust, high yield neural conversion of adult dermal fibroblasts. This vector demonstrated it can be used to efficiently convert fibroblasts collected at three different clinical sites from individuals with idiopathic as well as genetic forms of Parkinson's disease and Alzheimer's disease as well as patients with Huntington's disease. This new approach to iN conversion has great potential for disease modeling, diagnostics and drug screening and discovery across a range of neurological disorders that develop later in life – a set of conditions that have to date been nearly impossible to model using this approach.

Inventor Profile

Dr. Malin Parmar, Ph.D., is a NYSCF-Robertson Stem Cell Investigator and Professor at Lund University, Sweden, where she is focusing on bringing new cell-based therapies for Parkinson's disease to the clinic by replacing lost dopamine neurons with new, healthy cells. Dr. Parmar's work in cellular reprogramming opens up the possibilities of personalized treatments of patients with healthy versions of their own cells. Dr. Parmar completed her postdoctoral studies at Lund University, Sweden and Edinburgh University, Scotland.