Individualized Cell Therapy for Parkinsons Disease

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Country

USA

Title of project or programme

Individualized Cell Therapy for Parkinsons Disease

Source of funding information

NIH (NINDS)

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€ 2,799,426.61

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01/06/2012

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1

The project/programme is most relevant to:

Parkinson's disease & PD-related disorders

Keywords

dopaminergic neuron, Cell Therapy, Parkinson Disease, induced pluripotent stem cell, Midbrain structure

Research Abstract
DESCRIPTION (provided by applicant): Parkinson’s disease (PD) results from degeneration of midbrain dopamine (DA) neurons and can be effectively treated with L-dopa in the initial phase.

However, DA supplementation does not halt the DA neuron degeneration process, nor does it correct the loss of DA neurons. Consequently, PD patients almost invariably lose responsiveness to L-dopa treatment over time. Transplantation of human fetal mesencephalic tissues to replace the lost DA neurons has shown efficacy in alleviating symptoms of some PD patients. This therapy, however, depends on collection of tissues from multiple fetuses of particular ages for a single patient, which makes it impractical for general application and is ethically problematic. This proposal explores the possibility of future personalized cell therapy for PD using a non-human primate model. We will derive safe and functional DA neurons from the skin tissue of individual Parkinsonian rhesus monkeys through generation of induced pluripotent stem cells (iPSCs) that are free of virus and transgenes and using our newly developed strategy for midbrain DA neuron differentiation. We will then label the cell genetically and transplant the midbrain DA neurons back to the monkey from which the cells are derived, and assess whether the DA neurons survive and contribute to therapy in a short term and whether the therapeutic outcome is sustained over a long term (2-3 years). Results from this study will determine the safety and efficacy of autologous stem cell therapy for PD in primates, thus setting up a foundation for future clinical trials using reprogrammed human cells.

Lay Summary
This study will derive dopamine nerve cells from monkey’s skin tissue and transplant the nerve cells back to the Parkinson’s monkey to determine if such autologous cell therapy will lead to long lasting improvement of movement deficits. This study mimics and sets up a foundation for future personalized therapy using reprogrammed human cells.

Further information available at:

Types:
Investments > €500k

Member States:
United States of America

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Parkinson's disease & PD-related disorders

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