Human pluripotent stem cell differentiation, safety and preparation for therapeutic transplantation in Huntington’s disease

https://www.neurodegenerationresearch.eu/survey/human-pluripotent-stem-cell-differentiation-safety-and-preparation-for-therapeutic-transplantation-in-huntington%c2%92s-disease/

Principal Investigators
Institution
Contact information of lead PI
Country

European Commission

Title of project or programme

Human pluripotent stem cell differentiation, safety and preparation for therapeutic transplantation in Huntington’s disease

Source of funding information

European Commission FP7-Seventh Framework Programme

Total sum awarded (Euro)

€ 6,000,000

Start date of award

01/10/2013

Total duration of award in years

4.0

The project/programme is most relevant to:

Parkinson's disease & PD-related disorders|Huntington's disease

Keywords

Research Abstract

This project will tackle the huge complexity of taking stem cell therapies to clinical application for neurodegenerative disease by focusing on selective differentiation of a single neuronal phenotype (medium spiny striatal neuron: MSN) for a single well-defined disease (Huntington’s: HD). Our consortium contains expertise in all elements required to drive this technology to the point of clinical delivery, including expertise in stem cell differentiation and control of proliferation; in vitro genetic, molecular, cellular and functional characterisation; preclinical assessment in both rodents and primates models of HD; GMP knowledge, development and

production; and clinical translation. Our clinical team includes world leaders in HD clinical trials, including fetal neural transplants and is well placed to design the translation process. We focus on human embryonic stem (hES) cells as our primary target for first-in-man proof-of-concept studies, as they are closest to clinical readiness. HD is the target disease as it provides both an excellent model relevant to a wide range of neurodegenerative conditions, and is a stringent test of the capacity of selectively differentiated stem cells to repair neural circuits. The starting point for the work is the existence within the consortium of three of the most advanced protocols to date for MSN differentiation, and a feature of our consortium is that the specificity of stem cell differentiation will be tested against primary fetal MSNs (current gold standard) at all stages of both in vitro and in vivo assessment. In order to maintain flexibility in an emerging ethical environment, we will develop induced pluripotent (hiPS) cells to the point of GMP validation as a second generation target to hESCs. This will build European infrastructure and capacity to deliver emergent stem cell therapies through the highest quality clinical trials into clinical practice in a broad range of human neurodegenerative diseases.

Lay Summary
Further information available at:

Types:
Investments > €500k

Member States:
European Commission

Diseases:
Huntington's disease, Parkinson's disease & PD-related disorders

Years:
2016

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N/A

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