Patient derived iPS cells for investigating pathogenetic mechanisms of brain diseases that cause movement disorders

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Country

Norway

Title of project or programme

Patient derived iPS cells for investigating pathogenetic mechanisms of brain diseases that cause movement disorders

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RCN

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€ 432,050

Start date of award

01/12/2013

Total duration of award in years

3

Keywords
Research Abstract

The use of patient-derived induced pluripotent stem (iPS) cells represents a breakthrough in investigating the pathophysiology of genetic neurological diseases (Dimos et al 2008, Siller et al 2013). The general aim of this project is to use iPS cells to establish a platform to study underlying disease mechanisms associated with monogenic neurological diseases. The focus in this project is on hereditary movement disorders, specifically forms of spinocerebellar ataxia

(SCA) and of dystonia that are linked to single gene mutations and that are represented in Norwegian and other European patient populations. These movement disorders severely affect quality of life, the underlying disease mechanisms are poorly understood, and their is currently no cure. By ge nerating iPS cells from patients suffering from these diseases and differentiating these into the principal neuron types that are affected, we will be able to investigate the molecular and cell physiological mechanisms of the disease in ways that are not possible in the patients themselves. The project involves a close collaboration between basic research laboratories (Oslo University Hospital/University of Oslo and major foreign universities) and clinical neurologists and neurogeneticists in Norway and a t major international centers for clinical movement disorder research. The project thereby stimulates strongly translational research at a national and international level. It also lays the groundwork for establishing a general iPS cell-based approach rel evant for elucidating brain disease mechanisms with a utility beyond the specific diseases focused on here.

Further information available at:

Types:
Investments < €500k

Member States:
Norway

Diseases:
N/A

Years:
2016

Database Categories:
N/A

Database Tags:
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