MESENCHYMAL STEM CELLS FOR MACHADO-JOSEPH DISEASE THERAPY

https://neurodegenerationresearch.eu/survey/mesenchymal-stem-cells-for-machado-joseph-disease-therapy/

Name of Fellow

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Funder

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Country

Portugal

Title of project/programme

MESENCHYMAL STEM CELLS FOR MACHADO-JOSEPH DISEASE THERAPY

Source of funding information

FCT

Total sum awarded (Euro)

€ 116,640

Start date of award

01/01/13

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6.0

The project/programme is most relevant to:

Spinocerebellar ataxia (SCA)

Keywords
Research Abstract

Mesenchymal stromal cells (MSCs) are an extremely promising tool for therapy of neurodegenerative disorders. However, this therapeutic approach has received limited attention with respect to spinocerebellar ataxias (SCAs). In the present study, we aim at investigating the potential of MSC systemic transplantation as a cellular therapy for Machado-Joseph

disease/spinocerebellar ataxia type 3 (MJD/SCA-3), the most prevalent genetically-inherited SCA worldwide. For this purpose, we will evaluate the engraftment and neuroprotective/neuroregenerative effects of wild-type MSC transplantation both in in vitro and in vivo models of MJD and further dissect its protective mechanisms. In addition, we will evaluate if the neuroprotective potential of MJD MSCs is impaired and whether gene correction produce enhanced positive outcomes. Moreover, treatment with granulocyte colony-stimulating factor (G-CSF) will be compared with MSC transplantation. We expect to provide evidence that BM-MSCs can alleviate MJD and become a good candidate for disease-modifying MJD therapies, so far inexistent.

Types:
Fellowships

Member States:
Portugal

Diseases:
Spinocerebellar ataxia (SCA)

Years:
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Database Categories:
N/A

Database Tags:
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