SILENCING MACHADO-JOSEPH DISEASE THROUGH THE SYSTEMIC ROUTE

https://neurodegenerationresearch.eu/survey/silencing-machado-joseph-disease-through-the-systemic-route/

Name of Fellow

RUI JORGE GONÇALVES PEREIRA NOBRE

Institution
Funder

FCT

Contact information of fellow
Country

Portugal

Title of project/programme

SILENCING MACHADO-JOSEPH DISEASE THROUGH THE SYSTEMIC ROUTE

Source of funding information

FCT

Total sum awarded (Euro)

€ 116,640

Start date of award

01/01/10

Total duration of award in years

6.0

The project/programme is most relevant to:

Spinocerebellar ataxia (SCA)

Keywords
Research Abstract

Our group has recently showed that allele-specific silencing of ataxin-3 significantly decreased the severity of the neuropathological abnormalities in a rat model of Machado-Joseph disease (MJD) upon intracranial injection of lentiviral vectors (Alves et al., 2008a). Although intracranial injection is a safe procedure there is a need for a less invasive system. The aim of this project is

to develop a system that will allow delivery of siRNA-based treatments to the brain by a vascular route. AAV9 vectors will be engineered to transduce neurons of the adult mouse. Silencing sequences, previously shown to efficiently abrogate MJD, will be cloned into AAV9 and its efficacy evaluated in animal models (Alves et al., 2008b). It is expected that this project will enable the development of a new gene delivery system for the treatment of MJD and constitute a proof of principle for systemic gene therapy of a wide range of neurodegenerative diseases.

Types:
Fellowships

Member States:
Portugal

Diseases:
Spinocerebellar ataxia (SCA)

Years:
2016

Database Categories:
N/A

Database Tags:
N/A