Development of a platform to generate clinical grade neural progenitors for transplantation in Huntington’s disease

https://neurodegenerationresearch.eu/survey/title-of-pidevelopment-of-a-platform-to-generate-clinical-grade-neural-progenitors-for-transplantation-in-huntingtons-disease/

Title of project or programme

Title of PI	Development of a platform to generate clinical grade neural progenitors for transplantation in Huntington’s disease

Principal Investigators of project/programme grant

Title	Forname	Surname	Institution	Country
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Address of institution of lead PI

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- United Kingdom

Source of funding information

Medical Research Council

Total sum awarded (Euro)

819668.32

Start date of award

15-12-2008

Total duration of award in months

36

The project/programme is most relevant to

- Huntington’s disease

Keywords

Research abstract in English

Transplantation of striatal progenitor cells that reconstitute missing DARPP-32 positive medium spiny

projection neurons is a major strategy under assessment and consideration for the treatment of Huntington’s disease (HD). The greatest single issue for development of this therapy concerns the availability of a renewable source of appropriately specified donor neural progenitor cells.

Currently, directed differentiation of human embryonic stem cells (hESCs) to neural progenitors has the greatest potential of meeting this requirement. This view is supported by key advances in basic research including, (i) progress in developing methodology for the derivation and propagation of hESC lines, and (ii) understanding of underlying mechanisms of forebrain development and neuronal sub-type specification that provide a rationale for the development of directed differentiation protocols. Critically we have now developed protocols for neural differentiation under defined conditions to generate progenitors that express markers of forebrain fate determination, and that differentiate to DARPP-32 positive neurons both in vitro and in vivo in animal models of HD.

This translational project aims to expedite the ultimate goal of bringing stem cell therapies to clinical trial by adapting, and further developing, protocols to generate cells with markedly improved yields and a level of reproducibility and quality control that meet regulatory standards of good manufacturing practice (GMP), while maintaining a high functional efficacy in vivo in animal models of HD.

Lay summary
In which category does this research fall?

- Basic research