A trial to assess the safety and efficacy of intermittent putamenal GDNF infusions administered via Convection Enhanced Delivery (CED) in Parkinson’s

https://neurodegenerationresearch.eu/survey/a-trial-to-assess-the-safety-and-efficacy-of-intermittent-putamenal-gdnf-infusions-administered-via-convection-enhanced-delivery-ced-in-parkinsons/

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Country

United Kingdom

Title of project or programme

A trial to assess the safety and efficacy of intermittent putamenal GDNF infusions administered via Convection Enhanced Delivery (CED) in Parkinson's

Source of funding information

Parkinson's UK

Total sum awarded (Euro)

€ 2,772,172

Start date of award

29/10/2012

Total duration of award in years

4.0

The project/programme is most relevant to:

Parkinson's disease & PD-related disorders

Keywords
Research Abstract

Do intermittent infusions of glial cell line-derived neurotrophic factor (GDNF) promote neurorestoration in Parkinson’s.
Aim: To evaluate in Parkinson’s the efficacy and safety of intermittent GDNF intra-putaminal infusions administered by an improved Convection Enhanced Delivery approach permitting delivery to the entire posterior putamen.
Objectives: To assess the effects of intermittent GDNF infusions upon: off state motor symptoms; motor complications, cognition, mood, quality of life and 18F-dopa PET imaging. To determine the volume of distribution of GDNF post CED using MRI. To demonstrate the safety of GDNF infusions in
Parkinson’s, including GDNF antibody response.
Design: A single centre, randomized, double blind, placebo-controlled trial, in idiopathic Parkinson’s, of intermittent bilateral posterior putamen GDNF infusions administered via CED.
Methodology: 36 PD patients of moderate severity will be randomized in a 1:1 allocation to receive fortnightly infusions of GDNF or placebo for 9 months. Over the following 6 months (extension-phase) all patients will receive GDNF infusions every four weeks. Blinding will be maintained until the extensionphase ends. Primary and secondary clinical outcome measures will be performed at 8-week intervals. 18F-dopa PET imaging will be acquired at baseline, 9 and 15 months. Volume of distribution of infusate will be determined by serial MRI imaging. Safety and tolerability data will be recorded. The primary outcome measure is: Percentage change from baseline in the practically defined off state motor UPDRS score (part III) after 9 months of treatment.
Implications: Results from this trial will inform a rapid follow-on phase III multi-centre international trial.

Lay Summary
Further information available at:

Types:
Investments > €500k

Member States:
United Kingdom

Diseases:
Parkinson's disease & PD-related disorders

Years:
2016

Database Categories:
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Database Tags:
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