Conservative iron chelation as a disease-modifying strategy in Parkinson’s disease: a multicentric, parallel-group, placebo-controlled, randomized clinical trial of deferiprone

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Principal Investigators
Institution
Contact information of lead PI
Country

European Commission

Title of project or programme

Conservative iron chelation as a disease-modifying strategy in Parkinson’s disease: a multicentric, parallel-group, placebo-controlled, randomized clinical trial of deferiprone

Source of funding information

European Commission Horizon 2020

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€ 5,998,994

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01/05/2015

Total duration of award in years

5.0

The project/programme is most relevant to:

Parkinson's disease & PD-related disorders

Keywords
Research Abstract
Parkinson’s disease (PD) is a major, chronic, non-communicable disease and the 2nd most frequent neurodegenerative disorder worldwide. Excess iron is primarily detected in the

substantia nigra pars compacta, where dopaminergic neurons are exposed to high levels of oxidative stress produced by mitochondrial disorders and dopamine metabolism. Our previous preclinical, translational and pilot clinical studies demonstrated that novel iron chelation therapy with the prototypic drug deferiprone (DFP) (i) induces neuroprotection in cell models of PD via a powerful antioxidant effect, (ii) reduces regional siderosis of the brain, (iii) reduces motor handicap via inhibition of catechol-o-methyl transferase, and (iv) slows the progression of motor handicap in the 1-methyl-4-phenyl-1,2,3,6-tetrahydropyridine mouse model and in early PD patients. This project now seeks to demonstrate that conservative iron chelation therapy with moderate-dose DFP (30 mg/kg/day) slows the progression of handicap in de novo PD patients while not affecting systemic parameters. The 9-month, parallel-group, randomized, placebo-controlled, multicentre trial will be followed by a 1-month wash-out period. The primary efficacy criterion will be the change in motor and non-motor handicap scores on the Total Movement Disorders Society Unified Parkinson’s Disease Rating Scale to identify disease-modifying and symptomatic effects. The secondary efficacy criterion will be the change in score between baseline and 40 weeks (i.e. probing the disease-modifying effect only). Potential surrogate radiological and biological biomarkers, health economics and societal impacts will be assessed. 17 national, European and international research and innovation activities will be linked with the project. The study results should prompt academic and industrial research on iron chelation as a disease-modifying treatment in neurodegenerative diseases.

Lay Summary
Further information available at:

Types:
Investments > €500k

Member States:
European Commission

Diseases:
Parkinson's disease & PD-related disorders

Years:
2016

Database Categories:
N/A

Database Tags:
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