From Brain Gene Transfer Towards Gene Therapy: Pharmacological Assessment of AAV, CAV and LVV

https://neurodegenerationresearch.eu/survey/from-brain-gene-transfer-towards-gene-therapy-pharmacological-assessment-of-aav-cav-and-lvv/

Name of Fellow Institution Funder

European Commission FP7-Seventh Framework Programme

Contact information of fellow Country

EC

Title of project/programme

From Brain Gene Transfer Towards Gene Therapy: Pharmacological Assessment of AAV, CAV and LVV

Source of funding information

European Commission FP7-Seventh Framework Programme

Total sum awarded (Euro)

€ 1,597,783

Start date of award

01/11/12

Total duration of award in years

4.0

The project/programme is most relevant to:

Parkinson's disease & PD-related disorders

Keywords

Health sciences | gene vectors | brain gene therapy

Research Abstract

BRAINVECTORS aims devising new gene therapy(GT)-based treatments for Parkinson's and

other neurodegenerative diseases, in substitution of current systemic treatments, by delivering neurotrophic factors (GDNF) into the CNS with new vectors derived from adeno-associated (AAV), canine adenoviruses (CAV) and lentiviruses (LV) with inducible gene expression. Although AAV, CAV and LVV are considered acceptable in terms of bio-safety, their immune response must be well characterized in order to further develop these vectors for clinical trials. Furthermore, the possibility to switch-off the expression of neurotrophic factors in case of adverse effects represents a significant pharmacological progress of the gene therapy approach for Parkinson's disease. BRAINVECTORS will:

devise new inducible gene expression cassettes with increased sensitivity of transactivators and inducers reducing thus the dose of drugs necessary to obtain GDNF expression in brain;
characterise the immune responses induced by the components of GDNF-AAV, -CAV and -LVV in rodent models for Parkinson's disease by using biomarker-based immunological screening.

The project is based upon a network of 12 participants of public academic institutions and private non-profit organisations and SMEs in France, Germany, Italy, Netherland, Portugal, Spain, Sweden and Switzerland. Some of them are traditionally linked together in developing vectors backbones, vector production technologies and Parkinson's animal models. Others have strong immunological background, pioneering the biomarkers-based immuno-technologies for GT vectors, and have R&D expertise/facilities on/for animal cell technologies cGMP for biopharmaceuticals. 142 p-m will be involved during 48 months in R&D and TOK activities with 72 p-m of recruited postdocs and 70 p-m of seconded staff.

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Fellowships

Member States:

N/A

Diseases:

Parkinson's disease & PD-related disorders

Years:

2016

Database Categories:

N/A

Database Tags:

N/A