

MASTER'S DEGREE IN BIOMEDICAL RESEARCH Research Project Proposal

Academic year 2023-2024

Project Nº 37

Title: Identification of new therapeutic targets for Parkinson disease

Department/Laboratory

Therapeutic genes for neurodegenerative diseases Lab; Gene Therapy for Neurological Disease Program, Lab 2.06, CIMA.

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Summary

Parkinson's disease (PD) is the most common movement disorder, afflicting 1% of the population above the age of 65. Pathologically is characterized by the death of dopaminergic (DA) neurons within the pars compacta of the substantia nigra (SN). Current treatments focus on symptomatic management, and is urgent to identify effective neuroprotective or disease-modifying treatments that can halt PD progression. The lack of effective disease-modifying treatments for neurogenerative disease may relate to the complex neurobiology underpinning neurodegenerative processes and the absence of true translational animal models. In the context of PD, recently it has been developed an advanced model of PD based in the progressive accumulation of neuromelanin in dopaminergic neurons of the SN. The model is generated by injection of an adeno associated virus encoding the human tyrosinase in the SN. In our laboratory we have important preliminary data using this new PD model, both at behavioural and molecular level, confirming that it recapitulates the most important features of PD in humans. Thus, in this project we will take advantage of this new model and we will use comparative transcriptomics between disease and control conditions to identify the genes involved in the neurodegeneration of dopaminergic neurons. The identified genes will be considered as potential therapeutic targets by developing new therapies based in gene therapy that will be tested preclinically in the same model.

yes X no

Does the project include the possibility of supervised animal manipulation to complete the training for animal manipulator?