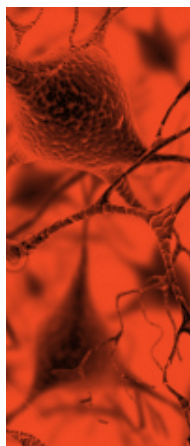


## REPark : Modeling Parkinson's disease by iPS technology: generation of human affected dopaminergic neurons and gene disease correction by site-specific integration

Austria Canada Finland **France** **Germany** **Italy** Israel Luxemburg Poland Romania **Spain**

**Project Description** Parkinson's disease (PD) is a disorder of old age with characteristic impairments of movement. Ever increasing numbers of PD in our aging society constitute a major burden to the health systems.

To develop new medications, it is critical to gain a full understanding of the cellular and molecular mechanisms of PD. However, despite a tremendous wealth of new information about the molecular basis of the disease, the lack of faithful cellular and animal models is delaying the development of new therapeutics. Thus, we are going to employ an innovative technology which uses patient skin cells and genetically reprograms them into brain nerve cells where different stages of disease progression and therapeutics can be studied over time. We plan to use an induced Pluripotent Stem cell (iPS) approach to generate dopaminergic neurons, which contain the diverse genetic factors that triggered PD. This technology will provide us with unlimited amounts of viable human cells derived from sporadic or monogenic PD patients which can be differentiated into electrically active nerve cells where the disease vulnerability can be studied during life. We plan to validate this pioneering in vitro system and to identify the cellular and molecular dysfunctions induced by PD mutations.



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