European Project on Mendelian Forms of Parkinson's Disease (MEFOPA)

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Title of project or programme

European Project on Mendelian Forms of Parkinson's Disease (MEFOPA)

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Research abstract in English

The Collaborative Project on Mendelian Forms of Parkinson?s Disease (MEFOPA) will bring together the major groups in Europe with a track-record in basic and clinical research on rare Mendelian forms of Parkinson?s disease (PD) in order to identify and validate relevant disease-related molecular pathways, drug-targets and biomarkers for disease susceptibility and progression.. Over the last years it has become increasingly clear that progress in the understanding of the molecular basis of PD, the second most common neurodegenerative disorder, and hence the chance to develop effective disease-modifying treatments, will most likely be brought about by focusing on the rare variants of the disease with known genetic defects. The groups forming the MEFOPA-consortium will therefore analyze the molecular pathways underlying inherited forms of PD with autosomal-dominant and autosomal-recessive inheritance in an integrative way, using cellular and animal models and cutting-edge technology. These two subprojects will provide targets for novel, disease-modifying treatment strategies. In a third subproject, a European registry and biobank for patients with rare Mendelian forms of PD will be established. Body fluids will be collected and systematically analyzed by unbiased proteomic techniques as well as by focussed analysis of candidate proteins, and ex vivo cellular models will be generated, in order to allow validation of disease-related alterations detected in the models analyzed in subprojects 1 and 2. Through this integrated, translational approach combining basic and clinical research groups, the project aims to achieve measurable progress in defining the relevant targets and readouts for disease-modifying therapies and will set the stage for rationally designed drug trials in carefully selected groups of patients and even presymptomatic mutation carriers.

Lay summary