Researchers, clinicians, companies, patients associations: connecting medical science people to achieve effective clinical trials

**Part A: From genes to therapy – emerging concepts for precision medicine in Parkinson’s disease**

**ABSTRACT**

Genetics of PD provided first insight into the complexity of this most common neurodegenerative movement disorder and delineated relevant subgroups of patients, who share an underlying molecular pathology. Novel patient-based models from monogenic forms of PD allowed to dissect mechanisms of neurodegeneration and define a first entry point to screen for disease-modifying compounds for more targeted therapies. Using neurons derived from induced pluripotent stem cells we implemented a comprehensive high-troughput/high-content screening (HTS/HCS) platform for discovering neuroprotective compounds in monogenic forms of PD. Here we provide first promising results to identify mechanism-based interventions in subgroups of PD patients using advanced cellular models.

During the last years, there has been a rapid evolution in novel technologies, e.g. next generation sequencing technologies or device-assisted registrations of PD symptoms. This is linked with a dramatic increase in high quality data characterizing PD at different levels and enables novel strategies for patient stratification and identification of markers for therapeutic outcome, that can be translated into precision medicine approaches and clinical decision support. Here we provide evidence for genetic predictors of therapeutic outcome from deep brain stimulation in PD. These implementation of novel technologies allow for a more direct participation of patients in ongoing research and strengthen patient’s autonomy and responsibility for future research.

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**Part B: EUPATI & Patient involvement: here we are, where we want to be in the future**

**ABSTRACT**

Today patients are largely unaware about clinical trial, translational research, personalized medicine and their potential supportive role in their areas.

The ultimate goal of EUPATI is to provide education and training to increase the capacity and capability of patients to understand and contribute to medicines research and development & to become active partners with academia, industry, regulatory agencies and ethic committees.

Within this session, we will explain the objectives of EUPATI and what patients can do to be more involved in their own therapy options and how the patients input can be valuable.

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