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Abstract

To date, no causative treatments are available for Parkinson's disease (PD) as the fastest growing neurodegenerative disease. Previously failed trials did not account for the clinical and pathophysiological heterogeneity of PD. 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. 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. The 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.