Data Driven Paths to Advancing Precision Medicine Strategies for Parkinson’s Disease: Critical Path for Parkinson’s Consortium

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Objective
An update on the accomplishments of the Critical Path for Parkinson’s (CPP) Consortium.

Background
The pharmaceutical pipeline for Parkinson’s disease (PD) is rapidly expanding. Data sharing will facilitate decision-making during the development and review of medical products [1]. The CPP Consortium was launched in 2015 and is jointly funded by Parkinson’s UK and pharmaceutical industry members under the auspices of Critical Path Institute [2]. The goals of CPP are 1) to advance drug development tools for PD trials by integrating worldwide data from observational cohorts and RCTs into a unified database, and 2) to provide a framework for advancing the regulatory maturity of digital health technologies [3]. The long-term goal is to enable precision medicine-based strategies for future PD clinical trials in a data-driven manner under the advisement of global regulatory agencies.

Methods
Members of the pharmaceutical industry, regulatory agencies (FDA & EMA), leading academic experts, professional societies, and international patient advocacy groups work together to accomplish the goals of CPP.

Results
Progress to date includes the development of a PD patient-level database of >10,000 subjects (Figure 2), a model-based qualification opinion from the EMA for the use of Dopamine transporter (DAT) neuroimaging as an enrichment biomarker for early motor clinical trials [4], the release of a DAT-based trial simulator, submission to FDA and EMA for regulatory acceptance of a PD disease progression model, and creation of the Digital Drug Development Tools Initiative (3DT). 3DT leverages the WATCH-PD (Wearable Assessments In The Clinic and Home in PD) study, a multicenter, prospective, longitudinal study of PD progression in subjects with early, untreated PD to facilitate discussion and alignment with regulatory agencies on evidentiary considerations for digital assessments for drug development.

Conclusions
The precompetitive regulatory science focus of CPP promises to advance the regulatory maturity of key technologies that will measure signs and symptoms of PD of importance to patients and increase the probability of success in future PD therapeutic trials. Future strategies include expanding the CPP database with clinical, genetic, and neuroimaging and fluid biomarker data.

References

Figure 1. Overview of Critical Path of Parkinson’s (CPP)

Figure 2. Overview of CPP’s database and Drug Development Tools output.