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Disease progression models for Huntington's disease

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Abstract

Huntington's disease (HD) is a progressive, inherited neurodegenerative disorder characterized clinically by a variety of symptoms, including motor dysfunction, cognitive changes, and psychiatric disturbances. Previous studies have shown that these symptoms, as well as brain abnormalities, could be detectable as early as one to two decades prior to diagnosis. Standardized clinical rating scales can be used to measure and track the progression of HD, of which the most widely used is the Unified Huntington's disease Rating Scale (UHDRS). UHDRS is a collection of scales, tests and questionnaires that facilitates a comprehensive rating of HD severity and has been used as a major outcome measure for HD clinical trials. Longitudinal information about how the items of UHDRS change over time is necessary to understand the disease mechanism, and it is best assessed by disease progression modeling (DPM). DPM is an important tool to quantitatively assess disease progression and factors that might modify that progression. It can also help with understanding the disease mechanism and predicting disease diagnosis and progression. Combined with pharmacological response modeling, DPM could provide a framework to evaluate HD therapeutic treatments that potentially slow disease progression and delay and/or prevent disease onset. Currently, there are only a few HD disease progression models and they are limited by study scale, study duration and analysis method. The objective for this project is to develop and evaluate DPMs that quantitatively describe the longitudinal changes of UHDRS for premanifest and manifest HD.