LECTURE: ARE THE CURRENT OUTCOME PARAMETERS IN PD CLINICAL TRIALS CLINICALLY RELEVANT?

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In Parkinson's diseases (PD) clinical trials the outcome parameters are important for evaluation of treatment or intervention. The outcome parameters could be evaluated by the clinician or by the patient. Patient-reported outcome (PRO) instruments represent a self assessment of any dimension of health status by the patient without interfering of the clinician. These instruments are used by investigators, sponsors and regulatory agencies.
Minimum important difference (MID) is quantification of change observed in a PRO measure between 2 or more treatment groups meaning treatment benefit. Properties that are measured in evaluation of a PRO instrument are: reliability, validity, ability to detect change, interpretability.
Statistical significance could be achieved sometimes for very small changes (but unimportant for the patient), if the study is large enough.
The responder definitions should be defined a priori. It should be stated also the methods in which MIDs are estimated. Usually are two types of methods: 1.) the anchor-based, in which there is an evaluation of the change of the analysed parameter and an independent variable of clinician or the patient; 2.) distribution-based, in which MID is assessed by analyzing the scores.
When two groups are evaluated for treatment benefit, one approach is the use cumulative distribution function (CDF) of responses. The lecture will detail all theses aspects with practical examples.