

Title: Determination of Minimally Clinically Important Change in Early and Advanced PD

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Delineating minimally clinically important change (MCIC) in Parkinson's disease (PD) is important. A clinical trial may identify a small but statistically significant change in an outcome measure that may have little or no relevance as to whether a patient actually feels improved. In PD, the two most commonly employed primary efficacy measures are change in UPDRS scores in early disease and change in OFF time in patients with motor fluctuations. In early disease, clinical trials have arbitrarily defined responders as those improving by 20% or 30% in UPDRS motor or total scores, and others have considered an improvement of three or five points on the UPDRS motor score as clinically meaningful. Previously published results utilized trials that did not have a placebo control group and medication doses were being escalated over time. Both of these factors might potentially inflate the derived MCIC. In addition, the derived MCIC might be higher for more efficacious treatments. To date, there have been no publications that define the MCIC for change in OFF time in patients with motor fluctuations. Based on this, the specific aims of this study are 1) to define the MCIC for UPDRS scores in early disease by analyzing data from the TEMPO study and other available early PD clinical trials; 2) to compare results of our analysis of MCIC for UPDRS scores in early disease to previously published results based on more efficacious treatments with no control group; 3) to define the MCIC for OFF time in patients with motor fluctuations by analyzing data from the PRESTO study and other available fluctuator studies. These MCIC cutoffs will be important for the design and interpretation of clinical trials, as well as for patient care decisions for those with early and advanced PD.