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Early initiation of treatment in Parkinson's disease prevents patient-reported deteriorations, but what is gained?

The paper by Grosset *et al*[1] (***see p 465***) in this issue describes self-reported health in a ''real-life'' cohort of dopa-naïve people with Parkinson's disease (PD). Assessments using the Parkinson's Disease Questionnaire (PDQ)-39 at initial consultation and for up to 18 months thereafter suggest stable self-reported health among patients who were started on dopaminergic treatment, whereas those who remained dopa-naïve deteriorated.

These observations add valuable fuel to the discussion on when to start dopaminergic treatment in PD.[2 3] However, it remains to be determined whether initial benefits last in the long term, or if potential early-treatment drawbacks[3] will emerge. Furthermore, additional outcomes to those presented here will be required to clarify this issue.

Meanwhile, interpretation of the current findings calls for some caution. For example, exactly what deterioration(s) did the untreated group experience? Deteriorations of PDQ-39 domains exhibited effect sizes varying from small to large.[1] However, to appreciate these it must be clear what the scores are intended to measure and whether they are valid representations of those domains. Unfortunately, this does not seem to be the case for the PDQ-39 and similar rating scales for PD.[4] This issue is particularly relevant in view of emerging standards from the US Food and Drug Administration, which call for clear support regarding score validity to make claims based on patient-reported outcomes.[5]

Second, why did treated patients not improve? In contrast to the observations by Grosset *et al*,[1] recent trials of the same types of drugs in de novo PD have shown early and lasting clinician-reported and patient-reported improvements. One reason could relate to the usefulness of the PDQ-39 among people with relatively low (ie, better) scores, as information on its performance in early untreated PD seem to be lacking and studies have suggested ambiguousness with its responsiveness.[6] If this is the case, it can have serious implications regarding interpretation of PDQ-39 outcomes, leading to valuable treatments being discarded when, in fact, people do benefit from them.

The study by Grosset *et al* adds an important aspect to the debate regarding when to initiate dopaminergic treatment in PD and its long-term extension will provide additional valuable insights. However, it also illustrates problems associated with rating scale endpoints, to which close attention needs to be paid since study design and statistics cannot compensate for measurement problems.[7] Because measurement properties are sample dependent and not fixed scale characteristics, one remedy would be for investigators to routinely report information on reliability and validity of the data used in the analyses that the study inferences rest upon. Indeed, inclusion of such information in the report by Grosset *et al* would have aided interpretation of their findings and shed light on the performance of the PDQ-39 in early PD. If we take our patients and our studies seriously, we also need to be serious about our outcome measures. Unless rating scales are treated with full scientific rigour, advances in the clinical sciences will be hampered and opportunities to improve patient care may be lost.

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