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Title: Does your PRO sum it all up? Investigating the variability in item specific PRO effects using random item slopes regression

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Abstract

Patient Reported Outcomes (PRO) are commonly analyzed to demonstrate patient benefit of treatments. When doing so, the focus will be on PRO sum scores, comprised of all or a subset of items. These scores may then be used in psychometric evaluations (e.g. criterion or known-groups validity) and to evaluate treatment arm differences. Rarely, is it investigated whether the constituent items of a scale score show consistent validity coefficients, or treatment differences. This is despite the increasing use of PRO instruments in new settings, where the psychometric evidence for their use is limited.

We discuss the application of random item slope regression (Donnellan, Usami, & Murayama, 2023), an application of linear mixed effect models, to evaluate whether items show consistent effects to the sum score. Results from a simulation study are presented to demonstrate how the method identifies differential item effects. First, we show how the average item effect (fixed effect) is equivalent to the criterion association estimated for the sum score if all items have consistent criterion associations. Next, we show that the effects at the level of the sum score become inaccurate under differing degrees of item specific associations (increased item slope random effect). Finally, we demonstrate how trial design, disease or sample specific variables can be included in the model to help explain variance in item criterion associations.

The application of random slopes item analysis has the potential to significantly improve our understanding of nuanced PRO effects, and as a result, patient benefit.