Abstract:

Background: Patient reported outcomes (PRO) are widely used in quality of life (QOL) studies, patient-centered health outcomes research, and clinical trials. The importance of PRO has been advocated by health regulatory authorities including FDA, NIH, and EMA. Patient Reported Outcomes Measurement Information System (PROMIS®) is a collection of standardized measures of PROs using modern test theory Item Response Theory (IRT). In clinical trials, power estimation is crucial to avoid waste of resources. However, in clinical trials with PROs as endpoints, closed form formula using total/ average scores are routinely used for power estimation. We aim to fill this gap and estimate power in a two-arm clinical trials with PROMIS® measures as endpoints with IRT model, power is also compared to the closed form formula.
Methods: We conducted a series of simulations to study the IRT model power with validated PROMIS® measures and parameters controlling factors including: sample size, effect size, number of items, and missing data proportion.
Results: Our results showed that sample size, effect size, and number of items are important indicators of IRT power with PROMIS® measures endpoints in clinical trials. When effect size is small and when sample size is very limited (n=25 in each group), closed form formula underestimates power compared to the IRT model with the PROMIS® measures.
Conclusions: IRT based simulation should be used for power estimation in two-armed clinical trials with PROMIS® measures as endpoints, especially when there is small effect size or small sample size.