MAYO CLINIC

Number of QOL items in a scale versus number of timepoints… it's complicated!

Is number of items in a scale or number of timepoints more important for precise estimation of mean change from baseline? (NCCTG N08CB, Alliance A152306)

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BACKGROUND

- Patient-reported outcomes (PROs) are commonly administered in clinical trials prior to treatment and at regular intervals leading up to a primary timepoint of interest
- To limit patient and staff burden, PRO administration could be limited to only the timepoints needed for formal hypothesis testing
- However, PROs collected at regular intervals can also be used to address early dropout and provide more precise estimates at subsequent timepoints, particularly in the presence of patient dropout

OBJECTIVES

The goal of this simulation study was to explore whether number of items in the scale or number of timepoints of administration are more important in precise estimation.

METHODS

- A completed Alliance trial (NCCTG N08CB, NCT01515787) was selected based on inclusion of a multi-item scale (EORTC QLQ-CIPN20 sensory neuropathy) administered at baseline and once per cycle (cycle length=2 weeks)
- Data were pooled across arms due to no differences being observed in the parent protocol (Loprinzi et al., J Clin Onc 2014; N = 353)
- Bootstrap samples of 100 patients were selected with replacement among patients with a baseline and at least one post-baseline measurement
- Mean change from baseline at cycle 10 was computed using a contrast estimated from a general linear mixed model using various numbers of items and cycles (See Supp Table)
- Mean half-widths of 95% confidence intervals were tabulated per scenario with 1000 replicates

RESULTS

- Regardless of the number of items, the half-widths decreased with additional timepoints
- The value of each incremental timepoint was greater from 2-5 timepoints versus 6-10 timepoints
- Independent of number of timepoints, the half-widths decreased with additional items
- While results were consistent when averaged across number of items and cycles, heterogeneity was observed depending on which individual scenarios were considered

Figure 1. Simulation results averaged across number of EORTC QLQ-CIPN20 sensory neuropathy scale items and cycles.

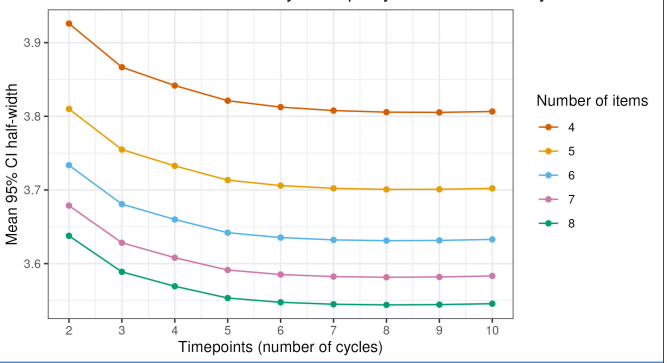
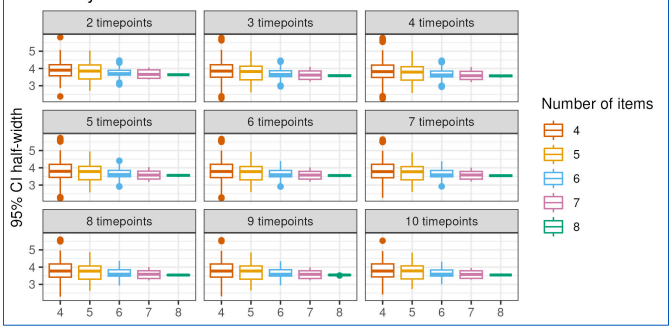


Figure 2. Distribution of simulation results for different combinations of number of EORTC QLQ-CIPN20 sensory neuropathy scale items and cycles.



CONCLUSION

- Results suggest that the relative importance of number of items versus timepoints is complex
- Impact on precision likely depends on various factors not manipulated in this trial dataset, including missing data rates, scale-level correlations across timepoints, and item-level correlations within a scale

https://acknowledgments.alliancefound.org http://DueckLab.github.io