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Reformulating PRO Estimators for Ordinal Outcomes in Oncology

LCP powering possibility

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Background

+ Patient-reported outcome (PRO) scores are commonly analysed as if they measure quantities, despite being based on ordered response categories.
+ Consequently, commonly used PRO estimators may rely on assumptions that are not justified by ordinal data alone.
+ Numerical score differences are often interpreted as having a consistent quantitative meaning across the full scale, despite this not necessarily being clinically or statistically defensible.
+ This study reviews the quantitative estimators used for PROs in recent oncology clinical trials and presents ordinally compatible alternatives that preserve the original clinical question

Objectives

+ Identify quantitative PRO estimators used in oncology clinical trials.
+ Examine the outcome-scale assumptions required to interpret these estimators.
+ Propose interpretable replacement estimators that do not treat score differences as quantities.

Methods

We reviewed **PubMed-indexed oncology clinical trials published between 2021 and June 2026**. Multiple PRO estimators could be extracted from a single trial. Estimators were independently classified by two reviewers, with discrepancies resolved through discussion. The unit of analysis was the reported estimator.

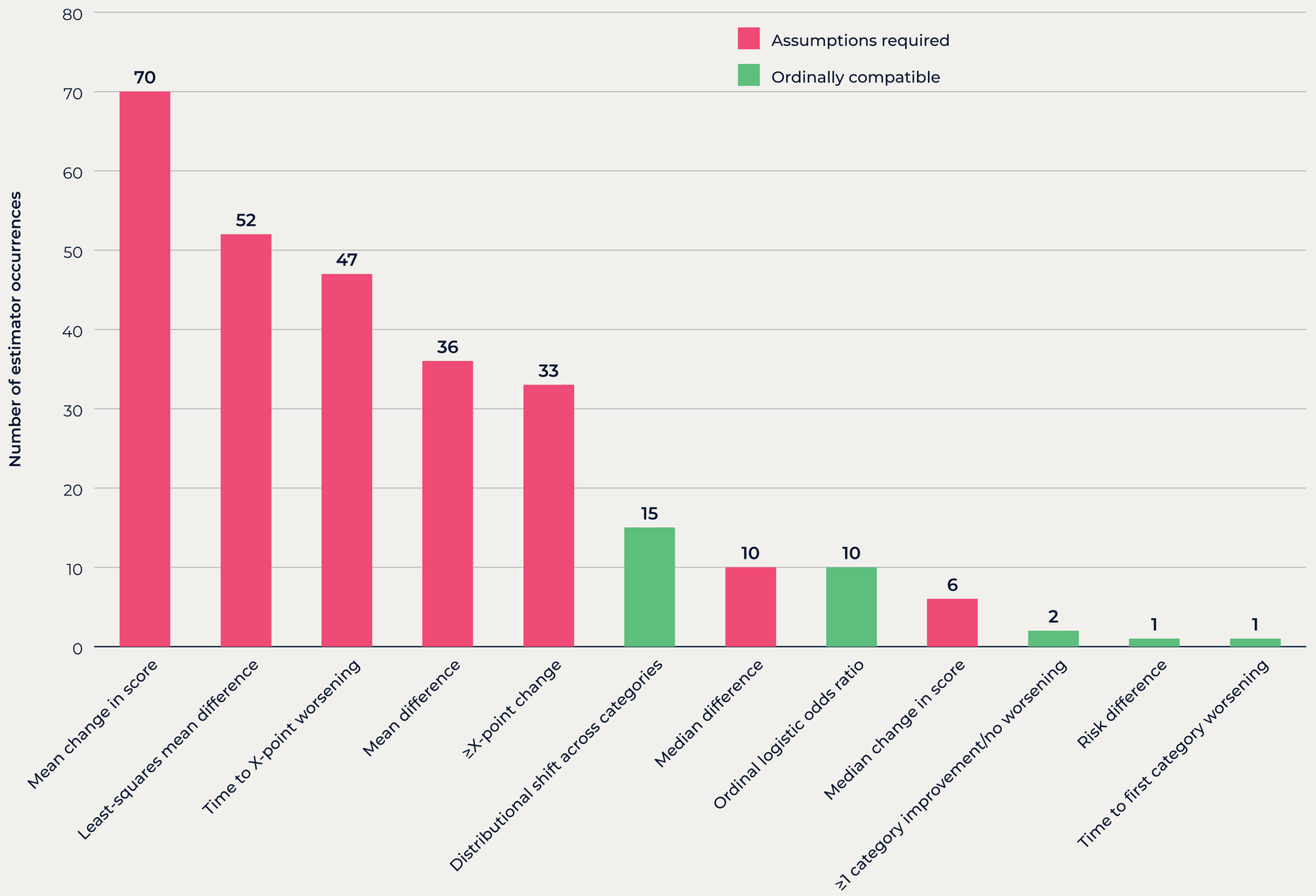
For each identified estimator, we:

+ Determined the implied outcome variable and required scale properties.
+ Assessed whether its interpretation was compatible with ordinal outcome data.
+ Reformulated the intended treatment comparison in terms of ordered categories.
+ Identified alternative estimators that retained the clinical meaning of the original question without requiring arbitrary numerical score distances to be interpreted quantitatively.

Results

+ A total of **283 PRO estimators** were identified with clearly defined analyses or methods, across **72 oncology clinical trials**. Of these, only **10.2%** of estimators were directly suitable for ordinal measures (green in Figure 1).

Figure 1: Frequency of PRO estimators reported in oncology clinical trials. Pink bars indicate estimators requiring assumptions beyond ordinal data alone; green bars indicate ordinally compatible estimators.



Why commonly used estimators can be problematic

+ **Mean change** and **least-squares mean differences** treat score differences as quantities. Their interpretation therefore requires score intervals to be meaningful and comparable.
+ **Fixed-point change** and **time-to-worsening** estimators assume that a given numerical change has the same clinical meaning regardless of baseline severity or location on the score range.

Table 1: Common estimators requiring additional scale assumptions and examples of ordinally compatible alternatives. Alternative estimators remain subject to their own modelling assumptions.

Common estimator	Ordinally appropriate alternative
Mean change in score	Probability of improvement, worsening or no change in ordered categories
Least-squares mean difference	Cumulative odds ratio (proportional odds model); marginal cumulative probabilities
Time to X-point worsening	Time to first worsening in an ordered severity category
Mean difference (between groups)	Marginal cumulative probabilities; cumulative odds estimators; probability that a treatment-group outcome is better than a comparator outcome
Fixed-point response	Category-based improvement; no worsening; attainment of a defined health state

In many cases, the underlying clinical objective can be retained while reformulating the estimator for ordered outcomes. Ordinally compatible approaches can preserve the intended treatment comparison while avoiding the need to interpret arbitrary numerical distances between PRO score categories as quantities.

Conclusions

Common oncology PRO estimators frequently require assumptions that are unsupported by ordinal data alone. Ordinally compatible estimators can answer the same clinical questions without treating numerical score differences as quantities. Their use can improve the interpretability and validity of analyses of PRO outcomes in oncology trials.

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