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A novel measure of treatment benefit for an ordinal scale: a case study of the IST-1 and the IST-3 stroke trials

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Background

Relative measures quantify the effect of an intervention but are difficult to translate into practice. Clinicians prefer absolute measures like the number needed to treat (NNT). We demonstrate a novel approach for reporting treatment effect across an ordered outcome where higher scores indicate worse functional outcome. We used the first and the third International Stroke Trials (IST-1 and IST-3) as case studies.

Methods

A two by 'K' table of treatment versus control over an ordinal outcome (with K levels) can be modelled as a multinomial distribution and the probabilities for each cell estimated. We calculated the number of score points gained per 1000 patients treated and estimated the 95% CI using bootstrap methods. Negative values indicate benefit with treatment whilst positive values indicate harm with treatment. We categorised patients into groups of poor functional outcome using prediction models (low (≤35%), medium (35 to 56%), and high (>56%)) and calculated the net gain in functional outcome within each stratum.

Results

The gain in Oxford Handicap Score (OHS) points per 1000 treated in IST-3 for low risk was 14 (95% CI -199 to 240), for medium risk -295 (95%CI -566 to -19) and for high risk -230 (95%CI -396 to -65). The gain in a four level functional outcome score in IST-1 for low risk was -112 (95% CI -214 to -9), for medium risk -42 (95% CI -95 to 12) and for high risk -29 (95% CI -59 to 2).

Conclusions

A 'net reduction in disability per 1000 patients treated' could be reported alongside the common odds ratio from the proportional odds model.

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