RESULTS: The binomial distribution, B(n,p), was used to model and simulate the probabilistic outcomes of therapy with the NNT and the absolute risk reduction (ARR) assigned to the trial size n and the event probability p, respectively. In this model, r out of n patients can prevent the adverse event with the probability P(x = r) according to any case for r = 0, 1, 2, ..., NNT as the size of NNT increases from one to larger numbers. Moreover, we developed the formula, as a function of NNT, to prevent the adverse event with the probability P(x = r) = \frac{\binom{n}{r} p^r (1-p)^{n-r}}{\sum_{k=0}^{NNT} \binom{n}{k} p^k (1-p)^{n-k}}

where p represents the probability of benefit, and NNT is the number needed to treat to prevent one adverse event. The formula can be simplified as:

\[ P(x = r) = \frac{\binom{n}{r} p^r (1-p)^{n-r}}{\sum_{k=0}^{NNT} \binom{n}{k} p^k (1-p)^{n-k}} \]

The factor analysis approach to item reduction produced a PTRQoL instrument with domains similar to the clinical impact approach. Measurement properties of the instrument developed using both methods may decide the optimal approach. Results from the 2 separate datasets and methods provide evidence for the robustness of the underlying conceptual framework of the PTRQoL.

PMI23

THE ODDS OF BENEFIT VERSUS NON-BENEFIT OF THERAPY ASSOCIATED WITH THE NUMBER NEEDED TO TREAT

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OBJECTIVE: To investigate one of the most common misunderstandings on the number needed to treat (NNT) that implies ‘certainly’, not statistically, one adverse event can be prevented if the patients of the NNT size are treated with the new therapy; to correct the wrong interpretation, we developed the odds method for the NNT to describe the benefit vs. non-benefit of therapy.

METHOD: The binomial distribution, B(n,p), was used to model and simulate the probabilistic outcomes of therapy with the NNT and the absolute risk reduction (ARR) assigned to the trial size n and the event probability p, respectively. In this model, r out of n patients can prevent the adverse event with the probability P(x = r) defined by B(n,p). We calculated the values of the P(x = r) according to any case for r = 0, 1, 2, ..., NNT as the size of NNT increases from one to larger numbers. Moreover, we developed the formula, as a function of NNT, to represent the odds of benefit vs. non-benefit of therapy, i.e., P(x ≥ 1)/P(x = 0).

RESULTS: The probabilities of non-benefit, i.e., P(x = 0), were between .25 and .40 for any size of NNT. It suggested the likelihood of non-benefit of therapy cannot be negligible even if the NNTs of small size seem to be ‘beneficial’. The numerical evaluation of the odds formula showed that the larger the sizes of NNT became, the smaller did the values of the odds. The progression over the NNTs of more than five was lower than 2.0, asymptotically converging to e-1 (i.e., about 1.7) as the size of NNT increases to infinity.

CONCLUSIONS: Although the NNT is a quite useful benchmark of the benefit of therapy, we must interpret it as carefully as possible with the odds that indicates the ‘relative’ benefit associated with the non-benefit of therapy.

PMI24

JUSTIFYING THE USE OF COST MINIMIZATION ANALYSIS: DEMONSTRATION OF COMPARATOR EQUIVALENCE

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Cost minimization analysis (CMA) requires equivalent comparators. Published guidelines have not adequately described criteria for assuring equivalence between pharmacotherapies.

OBJECTIVES: This study aimed to determine the proportion of published CMAs that provided evidence of equivalence between drug comparators.

METHODS: Medline, Embase, IPA, and Econlit databases were searched using text words “cost” and “minimization”. Inclusion criteria were: original research claiming to be a CMA that compared costs between drugs, and were published as full articles (i.e., not abstracts). Data extracted included: data demonstrating equivalence, drug class, and journal type. Adequacy of evidence was assessed by two raters and was based on source of evidence, quality and strength of effectiveness data, outcome of interest, and rater’s overall impression. Verification was through consensus.

RESULTS: The search identified 416 studies; 358 were rejected (272 did not compare drugs, 63 did not claim to be CMAs, 23 were abstracts); 12 were unavailable. Journals publishing the 46 accepted studies were: general medical (n = 24), hospital/pharmacy (n = 15), and health economics (n = 2). Based on adequacy criteria, 7 (15%) studies were judged “adequate”, 12 (26%) were “questionable”, and 27 (59%) “failed” to provide adequate evidence of equivalence. Of those studies judged “adequate”, drugs examined included: antibacterials (n = 4), cardiovascualrs (n-2), and antineoplastics (n = 1). Four of those seven were published in general medical journals.

CONCLUSION: The majority of studies failed to provide adequate evidence to justify using CMA as an analytic technique. Guidelines should be developed that explicitly specify criteria for CMA, and future authors should comply with those guidelines. Further study should examine CMAs not exclusively dealing with drugs and on studies that are CMAs but do not claim to be.