SESSION II

METHODOLOGY ISSUES—Clinical Outcomes Study Issues

PMD1

THE NEW METHOD FOR TIME ADJUSTMENT OF THE NUMBER NEEDED TO TREAT AND ITS APPLICATION TO PHARMACOECONOMICS ANALYSIS

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The number needed to treat (NNT) is a benchmark invented in evidence-based medicine to describe the incremental effect between two medical treatments. Although it is potentially useful on pharmacoeconomics, the estimate is limited to be valid at a certain endpoint of a clinical trial. **OBJECTIVE:** To overcome such time-constraint, we developed a new method that can standardize the NNT to ex/intrapolate over the time axis. **METHOD:** We reviewed a linear approximation model in which the absolute risk reduction (ARR), the reciprocal of the NNT, is directly proportional to the duration of the clinical trial. Then, we extended the linear model into a more complex model using exponential function to ex/interpolate the NNT, and also developed mathematically how to calculate the incremental cost-effectiveness ratio according to the standardized NNT. **RESULTS:** We found the linear model has a fatal disadvantage that the time-adjusted ARR can be larger than 1 (i.e., the time-adjusted NNT can become impractically smaller than 1) under the assumption of constant relative risk reduction and constant event rate over time. On the other hand, the exponential model was able to overcome such a disadvantage in consistency with practical assumptions. **CONCLUSION:** The exponential model is practically better than linear model to standardize the NNT. The method we developed is applicable to estimate the incremental cost-effectiveness introducing the ratio of incremental cost per person responded to a treatment.

PMD2

THE USE OF DISCRETE CHOICE MODELLING IN THE DESIGN OF CLINICAL TRIALS

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OBJECTIVE: Discrete choice modelling (DCM) applied to data generated by stated preference (SP) experiments is being used increasingly by health economists as a method for valuing process and outcome characteristics of health interventions in utility or monetary terms. The purpose of this paper is to illustrate how sponsors of randomised controlled trials (RCTs) could use DCM to assist with the planning of their studies. The approach is illustrated using a case study of the design of trials to evaluate the use of adjuvant bisphosphonates in the management of patients with primary operable breast cancer. **METHODS:** A stated preference experiment was conducted amongst UK specialists involved in the management of patients with primary operable breast cancer. Respondents were asked to choose 1 bisphosphonate regimen from each of 16 binary choice scenarios. Each regimen was characterised in terms of the following trial design attributes: 1) Primary endpoint; 2) Effect size demonstrated; 3) Uncertainty surrounding demonstrated effect; 4) Duration of observation; 5) Study population; and 6) Cost of the treatment alternatives. The survey was performed using a telephone-mail-telephone approach. Probit analysis was used to estimate a binary choice model of drug prescribing behaviour. **RESULTS:** 54 specialists fully completed the survey questionnaire providing a sample of 864 discrete choice responses. In qualitative terms, the signs on the coefficients were in line with prior expectations and all coefficients were statistically significant at conventional levels. The marginal and average effects were used to determine the relative importance of the attributes and to rank alternative designs in terms of the ex ante probabilities of product adoption. **CONCLUSIONS:** DCM could be used by sponsors of RCTs to incorporate decision-maker preferences into their designs. It could also be used to estimate product uptake contingent upon different designs. Results from this study suggest that the approach is both feasible and valid.

PMD3

RELEVANCE OF PATIENT REPORTED OUTCOMES FOR CHRONIC PAIN PATIENTS: THE ROLE OF SATISFACTION WITH ANALGESIC MEDICATION AND APPLICATION FORM

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OBJECTIVES: Patient satisfaction is a multi-facet construct and an important parameter of patient reported outcomes (PRO). In order to be able to measure chronic pain patient's satisfaction, the relevance of different dimensions were identified, with a focus on the role of analgesic medication. **METHOD:** First, a literature review was conducted to summarise the current instruments of PRO measures. To identify relevant dimensions of patient's satisfaction, 4 focus groups were held (10 patients with tumour pain, 10 with non-tumour pain). In semi-standardised interviews, patients' personal pain experience and articulation were explored, including their satisfaction with analgesics in different dosage forms (oral, transdermal). **RESULTS:** Besides HRQoL and other parameters, the PRO referred to the literature also include a reference to the importance of data relating to patient satisfaction. To date, instruments used to survey patient satisfaction have three dimensions: 1) Satisfaction with