NOT ALL PATIENTS ARE AVERAGE: THE IMPORTANCE OF RECOGNISING PATIENT HETEROGENEITY
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OBJECTIVES: Cost-effectiveness analyses are routinely based on data from group averages, restricting its generalizability to those with below- or above-average risk. A pharmaco-economic model was developed that used individualised risks, taking as example bisphosphonates and prevention of fractures. METHODS: Data were obtained from a research database of general practitioners, comprising a sample of the UK general population of women >50 years (N = 330,000). Individual mortality and hip, vertebral, and other osteoporotic fracture risks were estimated by age, sex, body mass index, smoking and other clinical risk factors. Estimates on costs, EQ5D utilities and treatment efficacy were obtained from a UK national report (NICE) and outcomes were simulated over a ten-year period. RESULTS: There was a large variability in the cost-effectiveness with clinical risk factors. At age 60–69, the cost per QALY gained was ≤136k in women with low fracture risk but ≥36k with high fracture risk (data for women without fracture history). Patients with low body mass index (<20) had considerable better cost-effectiveness than patients with high BMI (≥26) (≤23k versus ≤71k at age 60–79 in women without fracture history). The same was found for different diseases such as rheumatoid arthritis or inflammatory bowel disease. Using a cost-acceptability ratio of ≤30k per QALY gained, bisphosphonates became cost-effective for patients with a 5-year risk of 9.3% (95% CI 8.0–10.5%) for osteoporotic fractures and of 2.1% (95% CI 1.5–2.7%) for hip fractures. Including bone mineral density in the risk assessment, the cost per QALY gained was ≥35k in women at age 60 with a fracture history and a T-score of ~2.5 (at age 80, this was ≤3k). CONCLUSIONS: A pharmaco-economic model based on individual long-term risks (as derived from a health care database) can improve the targeting in a cost-effective manner of therapy to patients.

BAYESIAN ESTIMATION OF COST-EFFECTIVENESS ADJUSTED FOR REAL WORLD CONSIDERING STATISTICAL ERRORS IN CLINICAL TRIALS
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Although evidence-based treatment is a norm for practice, the efficacy of treatment presented in clinical trials does not necessarily guarantee its full size of benefit in a real world. One of the factors to be considered is statistical error, which may cause a discrepancy between “efficacy” in a clinical trial and “effectiveness” in real practice. OBJECTIVES: The purpose of this study is to formulate the influence on the cost-effectiveness in practice caused by statistical errors when a treatment is undertaken on the basis of the evidence proven in a clinical trial. METHODS: A decision analysis was performed by decision-tree modeling with treatment options that reflected the alternatives: take a new treatment A (TA) vs. a conventional treatment B (TB), depending on the evidence resulted from a clinical trial. The rule for selecting a treatment is: 1) take TA if the clinical trial confirmed better efficacy with TA than TB, or 2) keep using TB if failed to prove dominance of TA over TB. Also the decision tree modeled Bayesian prior probabilities at the chance node in the first depth with both the null hypothesis H0 and the alternative H1 (in efficacy, TA = TB, and TA > TB, respectively). RESULTS: Folding-back and averaging-out procedures in the decision-tree led to a mathematical formula with the parameters such as cost-effectiveness ratios, type I and type II errors, and Bayesian prior probabilities for treatment effect. Numerical analysis for the developed formula delineated an expected decrease of cost-effectiveness is not always negligible depending on the statistical power of the clinical trial and also on the degree of prior belief in a new treatment from the standpoint of Bayes. CONCLUSIONS: In case of need for adjusting cost-effectiveness from clinical trial into real practice, the formula newly developed can be a tool to measure the degree of the adjustment.

MEASURING SOCIAL PREFERENCES FOR EQ-5D HEALTH STATES: NEW SOLUTIONS TO OLD PROBLEMS
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OBJECTIVES: The standard version of EQ-5D defines 243 health states. Eliciting corresponding social preference weights is a labour/resource intensive exercise—witness the scale of the original 1993 UK valuation study and the subsequent US replication study. This stems from the requirement to use TTO methods in face-face interviews. Furthermore, given the need to minimise respondent burden only a subset of 45 health states are directly valued. These data are used to construct an estimation model used to interpolate values for the remaining health states. The complexity of this modelling represents a daunting challenge. Simpler methods are called for. This paper reports on a study in which values for all EQ-5D health states were obtained directly. METHODS: The EuroQoL Group has a standard questionnaire for use in collecting valuations in postal surveys. Multiple versions of this questionnaire were designed, each presenting a subset of 16 EQ-5D health states drawn from across the severity range. Values for health states are assessed on a 0–100 scale that represents worst-best imaginable health. A total of 1100 questionnaires were mailed to a sample of respondents selected from the electoral registers of England and Wales. Data from 685 respondents were subsequently analysed. RESULTS: OLS regression was used to determine the value decrements associated with increased problems on each EQ-5D dimension. The adjusted R² for this model is 0.907. Mean values from this survey were compared with mean VAS scores elicited in the original 1993 survey. For 30/45 health states values were remarkably associated with increased problems on each EQ-5D dimension. The standard version of EQ-5D defines 243 health states. Eliciting corresponding social preference weights is a labour/resource intensive exercise—witness the scale of the original 1993 UK valuation study and the subsequent US replication study. This stems from the requirement to use TTO methods in face-face interviews. Furthermore, given the need to minimise respondent burden only a subset of 45 health states are directly valued. These data are used to construct an estimation model used to interpolate values for the remaining health states. The complexity of this modelling represents a daunting challenge. Simpler methods are called for. This paper reports on a study in which values for all EQ-5D health states were obtained directly. METHODS: The EuroQoL Group has a standard questionnaire for use in collecting valuations in postal surveys. Multiple versions of this questionnaire were designed, each presenting a subset of 16 EQ-5D health states drawn from across the severity range. Values for health states are assessed on a 0–100 scale that represents worst-best imaginable health. A total of 1100 questionnaires were mailed to a sample of respondents selected from the electoral registers of England and Wales. Data from 685 respondents were subsequently analysed. RESULTS: OLS regression was used to determine the value decrements associated with increased problems on each EQ-5D dimension. The adjusted R² for this model is 0.907. Mean values from this survey were compared with mean VAS scores elicited in the original 1993 survey. For 30/45 health states values were remarkably similar and differed by less than 5 points. The highest proportional difference occurs for dead with a value in 2003 that is 45% higher than that recorded in 1993. CONCLUSIONS: The direct valuation of EQ-5D health states is feasible and yields robust results that avoid the use of problematic TTO procedures.