Abstracts

(mCRC) was not recommended in the UK (until June 2009), but accepted under a price volume agreement scheme with prior authorization in Italy, and without restriction in France and Germany. These products are funded on top of DRG costs in France, but not in other countries. We reviewed how such differences might affect use of the TRZ in France and UK. METHODS: Data on drug utilization from June 2006 to July 2009 were extracted from the Synovate Oncology Monitor, an ongoing database tracking prescribing of anti-cancer therapies. Sample sizes varied between countries and indications, from 1700 to 6200 patients. RESULTS: Of patients receiving TRZ from July 08 to June 09 ranged from 9% (UK) to 16% (Italy) in early BC, 12% (Italy) to 19% (France) in first-line advanced BC and 10% (France) to 34% (Italy) in second-line (irrespective of HER2 screening). For CTX, utilization rates ranged from 0% (UK) to 11% (France) in first-line advanced BC and 19% (Italy) in second-line advanced BC. Utilization of TRZ increased over time in early stage BC. Utilization of CTX was stable, increased in Germany, and decreased in Italy. Dosages and patient profiles were comparable across countries.

CONCLUSIONS: Funding on top of DRG does not appear to increase drug uptake. Health technology policy involves much more than drug utilization alone. This study suggests that policy makers should consider other factors in their decision-making.

MULTICRITERIA DECISION ANALYSIS (MCDA) FOR DRUG COVERAGE DECISION BY A PUBLIC HEALTH PLAN: CASE STUDY OF TRAMADOL FOR CHRONIC NON-CANCER PAIN (CNCP) IN CANADA

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OBJECTIVES: To field-test a decision support framework (EVIDEM) and explore its utility for a provincial decision committee committee using tramatol for chronic non-cancer pain (CNCP) as a case study. METHODS: The EVIDEM framework includes a multicriteria decision analysis matrix (MCDA) composed of 15 quantifiable components of decision including six domains (disease impact, context of intervention, intervention outcomes, type of benefit, economics, utility and a qualitative tool including the involvement of the patients in decision-making). RESULTS: The committee estimated the value of tramadol for CNCP at 44% (min: 36%, max: 61%) of maximum value on the MCDA scale. Main contributors to the MCDA values were side effects of the medicine (15% of total disease severity) and percentage of patients affected by disease (15% of total disease severity) and impact on adverse event expenditures (8%). Limited improvement in efficacy, safety and patient reported outcomes were not significant contributors to MCDA value. For a majority of committee members, ethical considerations on utility, efficiency and fairness had respectively a positive, neutral and negative impact on the value of tramadon. CONCLUSIONS: By systematizing consideration of all components of decision and underlying evidence, the framework allows consistent approach to evaluating health care interventions. Further testing and validation is needed to advance MCDA approaches in health care decision-making.

PR1 INCORPORATING TARPIT-LEVEL UNCERTAINTY AROUND ESTIMATES FROM THE CATALOGUE OF EQ-SD SCORES

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OBJECTIVES: Analysts typically take as “fixed” the underlying tariff scoring function of a given utility instrument when conducting probabilistic sensitivity analysis, ignoring an important source of uncertainty. An “off-the-shelf” catalogue of EQ-SD scores from the population, has recently appeared. A comprehensive review of the current study aims to incorporate the uncertainty in the underlying U.S. EQ-SD tariff function by estimating confidence intervals around estimates from the catalogue. METHODS: The Medical Expenditure Panel Survey (MEPS), a general population survey in the U.S., was pooled (2001, 2002, 2003 and 2004) to create a sample of 79,524 adults with valid EQ-SD responses. Chronic conditions were classified by ICD-9 codes and Clinical Classification Category (CCC) codes. Censored least absolute deviations (CLAD) regression methods were used to estimate the marginal disutility of each condition controlling for age, comorbidity, gender, race, ethnicity, income and education. US tariffs for the EQ-SD (Shaw) were applied to questionnaire responses. However, instead of taking the US EQ-SD tariff as a “fixed” function of the questionnaire responses, 500 bootstraps were conducted drawing from a distribution of possible EQ-SD tariffs based on the standard errors from the original scoring estimation. RESULTS: A catalogue of marginal disutility (EQ-SD) scores for each chronic ICD-9 and CCC code were estimated 500 times based on the distribution of possible EQ-SD tariffs. The 95% range of these potential marginal disutilities is presented and compared. CONCLUSIONS: Scores and marginal disutilities for a wide variety of chronic ICD-9 and CCC codes can be used to estimate QALYs in cost-effectiveness analyses. This research provides a range of values around each marginal disutility in the catalogue of “off-the-shelf” EQ-SD scores. Uncertainty in the underlying US EQ-SD estimation tariff is incorporated in these ranges to encourage better understanding of the uncertainty in EQ-SD estimates and to facilitate future probabilistic sensitivity analyses.

HT4 PRAGMATIC CLINICAL TRIALS FOR DRUG APPROVAL: IS IT REALISTIC?

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BACKGROUND: Patients, clinicians, payers and policymakers increasingly are interested in understanding comparative “real world” effectiveness of pharmaceutical products, often noting that traditional clinical trials performed for regulatory approval do not address important questions about the full range of benefits and harms of new drugs used in typical clinical settings. While more pragmatic designs are used for post-marketing studies, to date, no group formally has considered their utility and feasibility for regulatory approval trials. METHODS: In 2009, the Center for Medical Technology Policy convened an expert stakeholder working group to characterize recurring gaps in evidence that generally are not addressed in regulatory trials, explore the potential to incorporate pragmatic trials into the regulatory approval process. RESULTS: The working group identified representative set of pragmatic trials that may be pragmatic, covering the engagement of post-regulatory decision makers early in the design process to methods for designing more efficient trials. CONCLUSIONS: The optimal approach to pragmatic trials may not involve incorporating all possible pragmatic features, as are typically associated with large, simple trials. Some domains of pragmatism are more important to payers than others and any incremental movement toward more pragmatic designs may be not only highly valuable, but feasible.

EVALUATING THE MEASUREMENT PROPERTIES OF AN AUGMENTED EQ-SD WITH THE INCLUSION OF TWO SINGLE QUALITY-OF-LIFE (QOL) INDICATORS USING THE MEDICAL EXPENDITURE PANEL SURVEY (MEPS)

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OBJECTIVES: To evaluate the measurement properties of the EQ-SD profile augmented with its 0-100 mm visual analogue scale (VAS) and a 5-point summary self-rated health (SRH). METHODS: We used data from 4,091 adults from the 2003 MEPS who had 21 of 7 most prevalent chronic conditions and completed the EQ-SD, VAS, and SRH. The original 101 VAS categories were collapsed into a 9-category item with sufficient responses in each category. Five SRH categories included “excellent”, “very good”, “good”, “fair” and “poor”. The Rasch rating scale and partial credit models were used to calibrate the EQ-SD and the single items, respectively. Calibrations were conducted using 4 different combinations: EQ-SD alone; EQ-SD+SRH; EQ-SD+VAS; and EQ-SD+SRH+VAS. Model goodness-of-fit was assessed in each disease group using INFIT mean squares (≤1.40). Principal Component Analysis of Rasch Residuals was used to confirm dimensionality examining the proportion of total variance explained by Rasch scale, person measures and item measures, respectively. RESULTS: Respondents were predominantly white, female, middle aged and suffered most commonly from hypertension (32%), diabetes (17%) and depression (15%). EQ-SD item “anxiety/depression” consistently showed misfit to the model across 7 conditions when EQ-SD was evaluated alone. The inclusion of VAS and/or SRH not only improved model fit, but also increased overall distribution of persons and items along the latent health trait. Specifically, within each disease model, both item were included, 4 groups showed good model fit (mean squares ≤1.40). Consistently across all groups, VAS captured more person measures while SRH captured more item measures. CONCLUSIONS: The EQ-SD’s measurement quality is enhanced by the inclusion of VASSR, which allowed for the assessment of self-valuations on health that are possibly overlooked by the EQ-SD. The EQ-SD+VAS+SRH may serve as a suitable measurement framework for deriving population preference-weights. Consequently, a new valuation algorithm is called for.

PODIUM SESSION I: RESEARCH ON PRO METHODS (INCLUDING UTILITIES)

NATIONAL CULTURE AND EQ-SD VALUE SETS

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Background: Despite the growing importance of the EQ-SD descriptive system as a basis for the valuation of QALYs in cost-utility analysis, for most countries there are no EQ-SD descriptive social value sets. Many researchers and policy makers wishing to use the EQ-SD descriptive system in a country for which there are no value set are advised to use one from a nearby or similar” population. Factors other than geographic proximity can affect the relative values of EQ-SD states. Objective This study explores the links...