PODIUM SESSION I: PATIENT REPORTED OUTCOMES I

PR1
RECOMPUTING VALUES FOR EQ-5D IN ACCORDANCE WITH NICE APPRAISAL GUIDANCE

Kind P
University of York, York, UK

OBJECTIVES: Measuring the QALY benefits of new health interventions is critically dependent on the values of the quality-adjustment index—putting the “Q” in QALYs. For many health-related quality of life indexes, including EQ-5D, there are multiple options when selecting the value set for use in a given application. For the purposes of health technology assessment in England and Wales the National Institute for Health and Clinical Excellence has published guidance that when conducting the appraisal of new health technologies the values of the relevant population should be applied. However, the standard set of values used to derive the EQ-5Dvals were collected from a national UK population survey that included Scottish respondents whose health care system is organised and funded separately from the NHS in England and Wales. This paper reworks the original UK survey data and presents a new set of weights for EQ-5D recalibrated using the appropriate population values.

METHODS: The Measurement and Valuation of Health (MVH) survey methods have been fully described elsewhere. A total of 3395 individuals used ranking, rating and TTO methods to value a subset of 15 EQ-5D health states. A total of 360 of these respondents were citizens of Scotland. RESULTS: Scottish respondents reported similar health status compared to their English counterparts. However Scottish TTO values were systematically higher for 23/26 mild-moderate states and 16/17 states worse than dead were lower than English values. The Scottish data were removed from the MVH dataset and a new OLS regression model was constructed ($r^2 = 0.492$). 70% of values for EQ-5D health states in the revised model differ by more than 0.05 when compared with the original MVH values currently applied by NICE. The impact of applying these weights varies with the severity of the condition under review. CONCLUSION: The use of existing MVH weights appears contraindicated if English QALYs are required.

PR2
NON-PERSISTENT USE OF ORAL ANTIDIABETIC DRUGS LEADS TO 20% DECREASED CHANCE OF HBA1C GOAL-ATTAINMENT IN DAILY CLINICAL PRACTICE

Koerselman J1, van der Bij S1, Erkens JA1, Kessabi S2, Groot MT2, Penning-van Beest FJA1, Herings RMC1

1PHARMO Institute, Utrecht, The Netherlands, 2Novartis Pharma AG, Basel, Switzerland

OBJECTIVES: Within the Dutch guidelines for treatment of Type 2 Diabetes Mellitus (T2DM), the target-value of HbA1c has been set at <7%. The aim of this study was to investigate the relationship between persistence with oral antidiabetic drug (OAD)-treatment and HbA1c goal-attainment in daily clinical practice.

METHODS: From the PHARMO record linkage system, comprising among others, linked drug-dispensing, clinical laboratory, and hospital data for >2.3 million subjects in The Netherlands, new users of OADs were identified in the period 1999–2005. Patients who started on monotherapy with metformin, a sulphonylurea (SU), or a thiazolidinedione (TZD), or on combination therapy with metformin+SU, or metformin+TZD, with baseline HbA1c >7% and at least one HbA1c-measurement in the period of 6–12 months after treatment-onset, were included in the study-cohort. Persistence with OAD-treatment in the first year of treatment was determined using the method of Catalan, and was defined as the duration of the first treatment-episode in days. In case the first treatment-episode overlapped with the date of a HbA1c-measurement, a patient was considered persistent at that measurement. Patients with HbA1c <7% were defined at goal.

RESULTS: The study cohort included 2023 patients. Three-quarters (1512 patients) were persistent with any OAD at the time of first HbA1c-measurement in the time-period of 6–12 months after OAD-start: of these, 861 (57%) were at goal. Of the 511 patients who were non-persistent with any OAD at that time, 239 (47%) were at goal. Non-persistent patients were about 20% less likely to be at goal (RR-adj: 0.82; 95% CI: 0.74–0.91), compared to persistent users of any OAD. CONCLUSION: Non-persistent use of OADs leads to a 20% decreased chance of HbA1c goal-attainment in daily clinical practice. This effect of non-persistence seems modest, but represents a very large number of patients, in whom OAD-use might be better controlled.

PR3
PATIENTS’, PARENTS’, AND PHYSICIANS’ RISK-BENEFIT TRADE-OFF PREFERENCES FOR CROHN’S DISEASE TREATMENTS

Johnson FR1, Ozdemir S1, Mansfield C1, Hauber AB1, Hass SL2, Siegel CA3, Sands BE4, Miller DW2

1RTI International, RTP, NC, USA, 2Elan Pharmaceuticals Inc, San Diego, CA, USA, 3Dartmouth-Hitchcock Medical Center, Lebanon, NH, USA, 4Massachusetts General Hospital, Boston, MA, USA

OBJECTIVES: Compare the risk-benefit preferences of patients, parents of juvenile patients and gastroenterologists for Crohn’s disease (CD) treatments.

METHODS: Panels of CD patients, parents of juvenile patients and gastroenterologists completed a series of choice-format conjoint trade-off tasks with varying efficacy and risk levels. Parents evaluated treatments for their child, gastroenterologists evaluated treatments for three patients (young, middle-aged, elderly). Treatment attributes included daily symptom severity and activity limitations, potential for CD complications, time between flare-ups, systemic steroid use, and three SAE mortality risks: serious infection (SI), progressive multifocal leukoencephalopathy (PML) and lymphoma.

Preference estimates were used to calculate the annual SAE-specific maximum acceptable risk (MAR) for various levels of clinical benefit. RESULTS: 342 patients, 105 parents of juvenile patients, and 315 gastroenterologists provided usable data for analysis. For all respondent groups improvement in daily symptom severity was the most important factor in treatment preferences and risk tolerance was greater for treatments with better clinical benefits. Physicians had lower MARs for young patients and higher MARs for the elderly. For middle-aged patients, gastroenterologists’ MAR ranged from 0.16% to 0.76% for death or disability from PML, 0.24% to 0.38% for death from SI, and 0.13% to 0.81% for death due to lymphoma. Patients’ preferences are similar to gastroenterologists’.
that of physicians’ preferences for middle-aged patients across most benefit levels. Compared to adult patients, parents have greater risk tolerance for treating severe CD symptoms, and smaller risk tolerance for treating moderate CD symptoms. CONCLUSION: Respondents indicated they are willing to accept defined mortality risks in exchange for clinical efficacy and that acceptance is affected by the degree of benefit, the patient’s characteristics and the nature of the SAE. Understanding risk-benefit preferences can assist in identifying appropriate treatments and in informing welfare-enhancing regulatory decisions.

BACK PAIN IN GERMANY: ARE THERE DIFFERENCES IN GUIDELINE INDEPENDENT AND PATIENT SELF-TREATMENT?

OBJECTIVES: Back pain has a considerable impact on HRQoL, even with new medications, treatments and guidelines the degree of suffering for patients is high. The objective was to compare HRQoL for patients with back pain in Germany for three different treatment groups (guideline-, non-guideline- and self-treatment-group). METHODS: Patients were consecutively recruited by physicians in general practice in 2005. Patients were categorized into the three groups according to pre-specified algorithms. All groups completed the generic SF-36, the disease specific FFBH, von Korff Index and PHQ-D questionnaires. In addition, a retrospective chart review was conducted. HRQoL data was compared between the groups. RESULTS: A total of 145 patients took part in this study (n = 29 guideline-group, 44 non-guideline-group, 72 with self-treatment). Patients in the self-treatment-group were younger than patients in guideline- or non-guideline-group (49.8 vs. 59.4 vs 57.4 years, p = 0.0021). The groups did not differ significantly in gender or other socio-demographic characteristics. The von Korff Index was lowest (i.e. poorer) in the self-treatment-group and highest in the guideline-group (p = 0.0077). Regarding SF-36, patients in the guideline-group had the lowest physical (30.2 ± 8.5) and mental (41.2 ± 13.5) component scores, only the differences regarding physical component were statistically significant between the groups (p < 0.0001), those regarding mental component were not (p = 0.2875). Regarding PHQ-D items, the groups did not differ in frequency of major depressive and other depressive symptoms. The guideline-group had significantly higher burden of somatoform symptoms compared to the other two groups (p = 0.0219). The self-treatment-group had the highest FFBH-R total score (67.7 ± 21.5) compared to other two groups (p = 0.0056). CONCLUSION: From the here collected data, it seems that patients who are treated according to guidelines have reached a higher degree of suffering (poorer HRQoL) compared to those patients with either treated without guidelines and/or treat themselves. Further research is warranted to confirm our findings.

PODIUM SESSION II: CANCER

CN1

USING THE FACT-NEUROTOXICITY TO EVALUATE QUALITY OF LIFE IN CANCER PATIENTS FROM ACROSS THE GLOBE

Eremenco S, Du H, Arnold B, Herzberg T, Cella D
Evanston Northwestern Healthcare, Evanston, IL, USA

OBJECTIVES: Translation of PRO measures is an essential component of research methodology in preparation for multinational clinical trials. The FACT-Neurotoxicity (FACT-Ntx) is used to evaluate the quality of life of cancer patients suffering from neurotoxicity, a side effect of certain treatments. This study set out to linguistically validate the FACT-Ntx for use in Denmark, India, Lithuania and South Africa. METHODS: The study sample consisted of 176 patients (96 males and 80 females), with varying cancer diagnoses and a mean age of 51 years, speaking 11 languages: Afrikaans (15), Danish (25), Gujarati (15), Hindi (15), Kannada (15), Lithuanian (15), Malayalam (15), Marathi (15), Punjabi (15), Tamil (15) and Telugu (16). The FACT-Ntx was translated according to standard FACIT methodology. Patients diagnosed with any stage cancer on any treatment and experiencing neurotoxicity completed the respective translated version and then participated in cognitive debriefing interviews. Statistical analyses (descriptive statistics, one-way ANOVA and reliability analyses) were performed on the quantitative data. Participant comments were analyzed qualitatively. RESULTS: The FACT-Ntx translations showed good reliability and linguistic validity. The internal consistency of all languages combined was 0.86, and all items correlated at an acceptable level. In general, the Ntx score differed across self-reported Performance Status Rating (PSR) groups (nonparametric Kruskal-Wallis test p < 0.0001). A nonparametric Generalized Linear Model (GLM) approach (with multiple comparison adjusted significance level 0.017) showed a difference between ‘PSR = 0’ and ‘PSR = 1’ (p = 0.0002) and a difference between ‘PSR = 0’ and ‘PSR = 2’ (p < 0.0001), both with ‘PSR = 0’ patients reporting less neurotoxicity. CONCLUSION: The FACT-Ntx has shown acceptable reliability and linguistic validity in 11 languages. The instrument also has shown adequate sensitivity in differentiating patients with no symptoms and normal activity from patients reporting some symptoms. We consider these translations acceptable for use in international research and clinical trials.

CN2

DEVELOPMENT AND VALIDATION OF OPTIMALLY WEIGHTED MEASURES OF GLOBAL HEALTH-RELATED QUALITY OF LIFE (QOL) AND UTILITY BASED ON A CANCER-SPECIFIC QOL INSTRUMENT

Grimison PS, Simes Rj, Stockler MR
NHMRC Clinical Trials Centre, University of Sydney, Camperdown, NSW, Australia

OBJECTIVES: To facilitate the comparison of net benefits of cancer treatments in clinical trials by developing and validating a system to convert data from a QoL instrument into precise and optimally weighted global QoL measures and utilities. METHODS: Two-hundred cancer patients completed the Utility-Based Questionnaire-Cancer (UBQ-C), a validated 34-item cancer-specific instrument which includes scales of health status, performance status: mean (95% CI) derived utility scores for ECOG 0-1 0.86 (0.77, 0.95), ECOG 2-3 0.35 (0.25, 0.44), ECOG 4 0.01 (0.00, 0.09). The FACT-Ntx scales showed good reliability and linguistic validity. The internal consistency of all languages combined was 0.86, and all items correlated at an acceptable level. In general, the Ntx score differed across self-reported Performance Status Rating (PSR) groups (nonparametric Kruskal-Wallis test p < 0.0001). A nonparametric Generalized Linear Model (GLM) approach (with multiple comparison adjusted significance level 0.017) showed a difference between ‘PSR = 0’ and ‘PSR = 1’ (p = 0.0002) and a difference between ‘PSR = 0’ and ‘PSR = 2’ (p < 0.0001), both with ‘PSR = 0’ patients reporting less neurotoxicity. CONCLUSION: The FACT-Ntx has shown acceptable reliability and linguistic validity in 11 languages. The instrument also has shown adequate sensitivity in differentiating patients with no symptoms and normal activity from patients reporting some symptoms. We consider these translations acceptable for use in international research and clinical trials.

CN1

USING THE FACT-NEUROTOXICITY TO EVALUATE QUALITY OF LIFE IN CANCER PATIENTS FROM ACROSS THE GLOBE

Eremenco S, Du H, Arnold B, Herzberg T, Cella D
Evanston Northwestern Healthcare, Evanston, IL, USA

OBJECTIVES: Translation of PRO measures is an essential component of research methodology in preparation for multinational