

Published Articles – Patient Reported Outcomes

Societal preference values for advanced melanoma health states in the United Kingdom and Australia.

Beusterien KM, Szabo SM, Kotapati S, Mukherjee J, Hoos A, Hersey P, Middleton MR, Levy AR.

British Journal of Cancer, 2009, 101(3)

ABSTRACT

BACKGROUND: No studies measure preference-based utilities in advanced melanoma that capture both intended clinical response and unintended toxicities associated with treatment.

METHODS: Using standard gamble, utilities were elicited from 140 respondents in the United Kingdom and Australia for 13 health states.

RESULTS: Preferences decreased with reduced treatment responsiveness and with increasing toxicity. **CONCLUSIONS:** These general population utilities can be incorporated into treatment-specific cost-effectiveness evaluations.

Impact of Lennox-Gastaut Syndrome (LGS) on health-related quality of life (HRQL) of patients and caregivers: literature review

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Seizure, 2009, 18

ABSTRACT

The aim of this study was to conduct a review of the literature to evaluate the impact of LGS on the HRQL of children with LGS and their caregivers. The literature search revealed that there is limited published research on the impact of LGS on the HRQL of the child or caregiver. LGS has a major physical impact on a child, with a high frequency of seizures, and a high rate of seizure-related injuries. It interferes with all aspects of the child's intellectual and social development. The patient, and also his/her entire family are affected. Caring for a child with LGS is described as a 'burden', with increased anxiety about injury from seizures as well as the strain associated with providing continuous care. Overall, there is a lack of research on LGS, specifically the extent of the impact of LGS on the HRQL of the child and caregiver is under-explored.

Reliability, validity and the ability to detect change of clinician rated Personal and Social performance scale in patients with acute symptoms of schizophrenia

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Current Medical Research and Opinion, 2009 25(2)

ABSTRACT

Objective: To describe the measurement properties of the Personal and Social Performance scale (PSP), a clinician-reported measure of severity of personal and social dysfunction, in subjects with acute symptoms of schizophrenia.

Methods: Pooled data from three paliperidone extended release clinical studies (n=1665) and data from a separate noninterventional, cross-sectional, validation study (n=299) were analyzed.

Results: The PSP showed good interrater (interclass correlation coefficient [ICC]=0.87) and test-retest (ICCs>0.90) reliability. Pearson correlation coefficient for association between baseline PSP and Positive and Negative Syndrome Scale (PANSS) total scores was -0.32 for subjects assessed by the same rater and -0.29 for subjects assessed by different raters, suggesting low overlap in measurement constructs between baseline PSP and Clinical Global Impression-Severity (CGI-S) scores was -0.51 with the same rater and -0.15 with different raters. Hypothesized relationships between the PSP and the PANSS or CGI-S based levels of disease severity were prospectively defined. These hypotheses were confirmed by analyses showing statistically significant differences between baseline mean. PSP scores in subjects grouped by severity rating on the CGI-S (mild or less vs. at least moderate) ($p<0.001$). Limitations of analyses include pooling data across studies, interrater reliability assessment in the validation study only, post hoc assessment of test-retest reliability in the paliperidone ER studies, different raters for the PSP and PANSS not specified in the paliperidone ER studies, PSP validity assessment based on the PANSS and the CGI-S as comparators rather than another social function instrument.

Conclusion: These initial reliability and validity assessments suggest the PSP has promise as a measure of social functioning in patients with acute symptoms of schizophrenia.

Psychometric evaluation of a patient-reported symptom assessment tool for adults with hemophilia (the HAEMO-SYM)

Haemophilia, 2009 15(5)

A Rentz E Flood C Altisent M Bullinger R Klamroth R P Garrido I Scharrer W Schramm E Gorina

ABSTRACT

In patients with hemophilia, repeated bleeding events result in significant comorbid conditions that can degrade health-related quality of life. Clinician-reported symptom measures are available for use in patients with hemophilia A or B; however, there has not been a validated patient-reported symptom evaluation instrument available for hemophilia to date. The objective of this study was to develop and evaluate a self-report instrument, the HAEMO-SYM, for measuring symptom severity in patients with hemophilia. Eighty-four hemophilic subjects from Canada and the USA were enrolled and completed the HAEMO-SYM, SF-36, and Health Assessment Questionnaire-Functional Disability Index (HAQ-FDI). Four-week reproducibility was evaluated in 72 stable subjects. Construct validity was assessed by correlating subscale scores with the SF-36, HAQ-FDI, a coping questionnaire and clinical scores. The final 17-item HAEMO-SYM has two subscales: pain and bleeds. Internal consistency reliability was good (Cronbach's alphas, 0.86-0.94) and test-retest reliability was good (Intraclass Correlation Coefficients, 0.75-0.94). HAEMO-SYM subscale scores were significantly correlated with SF-36 scores ($P < 0.05$ for all except HAEMO-SYM Pain and SF-36 Mental Health), HAQ-FDI scores ($P < 0.05$ for all but HAEMO-SYM Bleeds with HAQ-FDI Hygiene and Reach), Gilbert scale ($P < 0.01$), coping ($P < 0.05$) and global pain ($P < 0.001$).

Mean HAEMO-SYM scores varied significantly in groups defined by severity, HIV status and treatment regimen. Greater symptom severity was associated with more severe disease, HIV-positive status and prophylaxis treatment. The results of this study suggest that the HAEMO-SYM, a hemophilia-specific symptom severity instrument, has good reliability and provides evidence that supports construct validity in patients with hemophilia.

The impact of follicular lymphoma on health-related quality of life

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S Rule M Walker D Wild
Annals of Oncology, 2008, 19

ABSTRACT

Background: The purpose of this study was to determine whether there was a relationship between disease activity and health functioning, as measured by a range of patient-reported outcome (PRO) measures in patients with follicular lymphoma (FL).

Patients and methods: A total of 222 patients with FL were recruited from eight sites across the UK and they completed a number of PRO measures. The participants were analyzed across five disease states: 'active disease—newly diagnosed', 'active disease—relapsed', 'partial response', 'complete response' and 'disease free'. The relationship between these disease states and their level of health functioning was assessed as well as the relationship between being 'on' or 'off' chemotherapy and disease state.

Results: In terms of health-related quality of life (HRQoL), participants in the relapsed category had the lowest mean physical well-being, emotional well-being, functional well-being and social well-being score. In a regression analysis, the 'active disease—relapsed' group acted as a significant predictor for each PRO variable. In addition, the remission group acted as a significant predictor of high anxiety scores as measured by the Hospital Anxiety and Depression Scale.

Conclusion: The results of this study demonstrate that various aspects of patient-reported health outcomes differ according to disease state in patients with FL. For those patients who have relapsed, they are more likely to experience worse HRQoL and other patient-reported health outcomes than patients newly diagnosed, in partial or complete remission or when completely disease free.

Impact of schizophrenia and schizophrenia treatment-related adverse events on quality of life: direct utility elicitation

Health and Quality of Life Outcomes, 2008, 6(105)

A Briggs D Wild M Lees M Reaney S Dursun D Parry J Mukherjee

ABSTRACT

Objective: To examine the impact of schizophrenia, its treatment and treatment-related adverse events related to antipsychotics, on quality of life from the perspective of schizophrenia patients and laypersons.

Methods: Health state descriptions for stable schizophrenia, extra pyramidal symptoms (EPS), hyperprolactinemia, diabetes, weight gain and relapse were developed based on a review of the literature and expert opinion. The quality of life impact of each health state was elicited using a time trade-off instrument administered by interview to 49 stable

schizophrenia patients and 75 laypersons. Regression techniques were employed to examine the importance of subject characteristics on health-related utility scores.

Results: Patients and laypersons completed the interview in similar times. Stable schizophrenia had the highest mean utility (0.87 and 0.92 for laypersons and patients respectively), while relapse (0.48 and 0.60) had the lowest mean utility. Of the treatment-related adverse events, EPS had the lowest mean utility (0.57 and 0.72, respectively). Age, gender and PANSS score did not influence the utility results independently of health state. On average, patient utilities are 0.077 points higher than utilities derived from laypersons, although the ranking was similar between the two groups.

Conclusion: Events associated with schizophrenia and treatment of schizophrenia can bring about a significant detriment in patient quality of life, with relapse having the largest negative impact.

Results indicate that patients with stable schizophrenia are less willing to trade years of life to avoid schizophrenia-related symptoms compared to laypersons. Both sets of respondents showed equal ability to complete the questionnaire.

Psychometric evaluation of the Satisfaction with Oral Anti-Diabetic Agent Scale (SOADAS)

C Donatti D Wild R Horblyuk K Beusterien R T Anderson B Arondekar D B DiBenedetti
Diabetes research and clinical practice, 2008. 80

ABSTRACT

In this study we developed and evaluated the psychometric properties of the Satisfaction with Oral Anti-Diabetic Agent Scale (SOADAS), the first treatment satisfaction instrument specific to oral anti-diabetic agents (OADs). Three focus groups and three rounds of cognitive debriefing interviews with type 2 diabetes patients using OADs informed the development of the six-item SOADAS. The measurement properties of the SOADAS were evaluated in a US cross-sectional study.

Patients completed the SOADAS, Treatment Satisfaction for Medication Questionnaire (TSQM), the Short Form-12 (SF-12) Health Survey and a demographic questionnaire. The first half of the recruited patient population was asked to return at 7 days follow-up to complete the SOADAS again. An exploratory factor analysis on the SOADAS yielded a one-factor solution, indicating that the SOADAS was uni-dimensional. The SOADAS had high internal consistency reliability (Cronbach's alpha = 0.86) and acceptable test-retest reliability (ICC = 0.79). It was correlated most highly with the TSQM ($r = 0.71$, $p < 0.001$) and least with the mental summary component score of the SF-12 ($r = 0.24$, $p = 0.014$). Mean SOADAS scores were approximately two points higher for participants with lower HbA1c values (<7%) versus those with higher values, and for participants without versus with selected symptoms ($p < 0.05$).

The findings from this study suggest that the SOADAS is a valid and reliable measure of patient satisfaction with oral anti-diabetic medications. This questionnaire may be useful for assessing outcomes in future clinical studies of oral anti-diabetic medications.

Health state utility scores for cancer-related anaemia through societal and patient valuations

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Value in Health, 2008, 11(7)

ABSTRACT

OBJECTIVES: Fatigue is recognized as the most serious complication of chemotherapy for the majority of patients. This study aims to determine preferences and utility values for health state descriptions of anemia associated with cancer treatment. **METHODS:** FACT-An clinical trial data were summarized to define health states associated with hemoglobin levels of 7.0-8.0, 8.0-9.0, 9.0-10.0, 10.0-10.5, 10.5-11.0, 11.0-12.0, and 12.0+ g/dL. Health state descriptions were reviewed by clinicians and two quality-of-life experts. Eighty-five members of the general public were asked to rate the health states using a visual analogue scale and standard gamble (SG). Twenty-six oncology¹ patients were interviewed using the time trade-off (TTO).

RESULTS: The mean societal SG derived utility values showed a significant linear change from 0.583 +/- 0.067 (7-8 g/dL hemoglobin [Hb]) to 0.708 +/- 0.057 (12+ g/dL Hb). The patient TTO data ranged from 0.297 +/- 0.127 (7-8 g/dL Hb) to 0.611 +/- 0.092 (12+ g/dL Hb).

CONCLUSIONS: The health state utility scores from both groups show a decrement in line with worsening anemia. Furthermore, patients who have experienced cancer-related fatigue rate the more severe levels of anemia much lower than the general public.

Health state utility scores in advanced non-small cell lung cancer

S Doyle A Lloyd M Walker

Lung Cancer, 2008, 62(3)

ABSTRACT

BACKGROUND: The aim of the study was to capture societal utility scores for health state descriptions of advanced, metastatic non-small cell lung cancer (NSCLC), as well as determine disutility associated with specific disease symptoms.

METHODS: NSCLC health states were produced based on an adaptation of existing health state descriptions for metastatic lung cancer. The health states were expanded to contrast disease state (responding disease and stable disease) and impact of specific severe symptoms (cough; dyspnoea; pain; or no additional severe symptoms). Interviews with five lung cancer clinicians were carried out to assess the content and face validity of the existing health states as descriptions specific to NSCLC. The interviews also sought to explore the impact of the disease symptoms of interest. The resulting health states were reviewed by two psychometric experts independently. The final health states were piloted in a conventional standard gamble interview which revealed no significant issues in interpretation or comprehension. In the main study, 101 members of the general public assessed their preference for each health state in a chained standard gamble (SG) interview and on a visual analogue scale (VAS) rating scale. All participants also completed the EQ-5D and a socio-demographic form.

RESULTS: The study sample was a relatively good match to the characteristics of the general public in England and Wales. A mixed model analysis revealed that age, gender, and HRQL were not significant predictors of utility, but a treatment response and each of the disease symptoms were. When adjusted to match census data, stable disease with no additional symptoms had a utility value of 0.626. Health state values declined by 0.069 with the addition of pain; 0.050 for dyspnoea; or 0.046 for cough. A treatment response would result in a utility gain of 0.086.

CONCLUSIONS: Members of the general public showed a preference for responding disease over stable disease and a stable health state with no additional symptoms over a stable

health state with one of the three common NSCLC symptoms: cough, dyspnoea, and pain. The study highlights the value that society places on the avoidance of severe symptoms that some people with NSCLC can experience.

Health utility values associated with diabetic retinopathy

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Diabetic Medicine, 2008, 25(5)

ABSTRACT

AIMS: Different estimates exist regarding the impact of diabetic retinopathy (DR) on health utility. A previously reported prospective observational study has reported much larger decrements in self-reported utility than generic utility data from the UK Prospective Diabetes Study and the Lipids in Diabetes Study. The present study was designed to estimate utility loss using multiple methods.

METHODS: Detailed health state descriptions reflecting declining DR (five different visual acuity levels), neuropathy and nephropathy were validated with patients and used to elicit utility values from people with DR, people with diabetes and members of the UK general public using standard gamble. In addition, a larger sample of people with retinopathy completed different health-related quality of life measures in an interview [EuroQoL (EQ-5D), Health State Utilities Index (HUI)-3, and National Eye Institute Visual Functioning Questionnaire-25].

RESULTS: The utility scores from the standard gamble interviews were not significantly different between the three groups. There was a decline in utility from 6/6 vision to counting fingers of -0.244. The utility data derived from the generic measures revealed an equivalent decline of -0.41 on both the EQ-5D single index and the HUI-3.

CONCLUSIONS: This study has re-examined the utility decrements associated with DR and has identified much larger declines in utility than previously reported. The study has also reported the utility values of patients with retinopathy as assessed by standard gamble. We believe that this may be the first study to report utility values for health states associated with vision loss which have been elicited from patients with vision loss.

How does patient's quality of life guide their preferences regarding aspects of asthma therapy?

A J Lloyd E McIntosh A E Williams A Kaptein K F Rabe
Patient, 2008, 1

ABSTRACT

Background and objective: Previous work has suggested that people with poor health-related quality of life (HR-QOL) as a result of asthma are willing to pay the most for successful therapy. There is also evidence that preferences are an important influence on adherence to therapy. We report a patient preference study using a discrete-choice experiment (DCE) to elicit willingness to pay (WTP) of patients with asthma in Spain, the Netherlands, and the UK.

Methods: The DCE survey included different attributes of asthma therapy (days with symptoms, days needing reliever medication, asthma attacks [none, attack that does not

require doctor/emergency room {ER} visit, attack that requires doctor/ER visit], risk of adverse effects, number of preventer inhalers, and monthly out-of-pocket costs). Participants indicated which attribute combinations they preferred. The questionnaire survey included the mini Asthma Quality of Life Questionnaire (mAQLQ). The validity of the choice experiment has been supported by two pilot studies conducted in the UK. Preferences and WTP were estimated using a random effects probit model. Symptom days and asthma attack attributes were segmented by mAQLQ score.

Results: Demographic and clinical data were compared between the three country samples (UK, n = 124; Spain, n = 86; the Netherlands, n = 269). All study attributes were independently significant predictors of choice. People were willing to pay €35 per month to avoid a day with symptoms, and €109 per month (year 2005 values) to avoid experiencing asthma attacks that required emergency visits to their doctor or hospital. People with the worst HR-QOL were willing to pay the least to avoid days with symptoms and asthma attacks that required visits to their doctor/ER. This was not an income-related effect.

Conclusion: Patients who reported higher HR-QOL were willing to pay more to avoid days with symptoms and asthma attacks that required visits to their doctor/ER. Hypotheses were explored to explain this effect. It is possible that people with the least impairment of HR-QOL place the greatest value on avoiding the effects of asthma and so are most willing to alter their behaviour. People with the worst HR-QOL may be exhibiting a response shift type of effect whereby their internal values are shifted down by the burden of their disease. These findings may help to illuminate why there are differences in asthma treatment adherence.

The impact of chronic Hepatitis B on Quality of Life: A multinational study of utilities from infected and uninfected persons

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Value in Health, 2008, 11(3)

ABSTRACT

Objectives: Chronic hepatitis B (CHB) is a condition that results in substantial morbidity and mortality worldwide because of progressive liver damage. Investigators undertaking economic evaluations of new therapeutic agents require estimates of health-related quality of life (HRQOL). Recently, evidence has begun to accumulate that differences in cultural backgrounds have a quantifiable impact on perceptions of health. The objective was to elicit utilities for six health states that occur after infection with the hepatitis B virus from infected and uninfected respondents living in jurisdictions with low and with high CHB endemicity.

Methods: Standard gamble utilities were elicited from hepatitis patients and uninfected respondents using an interviewer-administered survey in the United States, Canada, United Kingdom, Spain, Hong Kong, and mainland China. Generalized linear models were used to the effect on utilities of current health, age and sex, jurisdiction and, for infected respondents, current disease state.

Results: The sample included 534 CHB-infected patients and 600 uninfected respondents. CHB and compensated cirrhosis had a moderate impact on HRQOL with utilities ranging from 0.68 to 0.80. Decompensated cirrhosis and hepatocellular carcinoma had a stronger impact with utilities ranging from 0.35 to 0.41. Significant variation was observed between countries, with both types of respondents in mainland China and Hong Kong reporting systematically lower utilities.

Conclusions: Health states related to CHB infection have substantial reductions in HRQOL and the utilities reported in this study provide valuable information for comparing new

treatment options. The observed intercountry differences suggest that economic evaluations may benefit from country-specific utility estimates. The extent that systematic intercountry differences in utilities hold true for other infectious and chronic diseases remains an open question and has considerable implications for the proper conduct and interpretation of economic evaluations.

Patient and caregiver perspective on paediatric eosinophilic esophagitis and newly developed symptom questionnaires

Current Medical Research and Opinion, 2008, 24(12)

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ABSTRACT

OBJECTIVE: Eosinophilic esophagitis (EE), a rare chronic inflammatory condition of the esophagus, is predominantly observed in children and is primarily manifested with feeding difficulties. To our knowledge, no self- or caregiver-reported questionnaires are available to assess pediatric EE symptoms and their impact as reported directly by children or their parents/caregivers. The objectives of this study were to characterize the symptoms and impact of EE among children as reported by patients and parents/caregivers and to assess the content validity of two newly developed pediatric eosinophilic esophagitis symptom questionnaires, one parent/caregiver-reported questionnaire for ages 2-7 years and one child-reported questionnaire for ages 8-17 years. The questionnaires were developed based on a review of the literature and clinical expert consultation.

RESEARCH DESIGN AND METHODS: This cross-sectional study involving one-on-one interviews with patients and caregivers was conducted at an American Partnership for Eosinophilic Disorders conference. Parents of patients aged 2-7 years (n = 12) and patients aged 8-17 years (n = 16) were first asked about symptoms and their impact on everyday life, using open-ended questions. Participants then completed the appropriate symptom questionnaire and were asked to provide feedback on the relevance, comprehensiveness, and clarity of each item and other questionnaire issues (time to complete, length, format, etc.). All reported symptoms were enumerated, and the feedback on the symptom questionnaires was analyzed qualitatively. **RESULTS:** The majority of study participants were white (82%) and male (86%). The most frequently reported symptoms of 2-7-year olds were vomiting (92%), "reflux" (50%), dysphagia (25%), abdominal pain (25%), and trouble sleeping (25%). The 8-17-year group reported abdominal pain (56%), vomiting (31%), throat pain (25%), diarrhea (25%), and food getting stuck (25%). Symptoms and treatment were reported to have a major impact on daily life, particularly on school, after-school activities and social events, feeling frustrated regarding symptoms and treatment, and feeling "different". Overall, participants thought that the questionnaires were clear, relevant, and appropriate for symptom assessment.

LIMITATION: This study was based on a small and convenient sample of participants attending an EE conference and hence may not be representative of the general EE patient population.

CONCLUSIONS: EE is associated with a range of symptoms that vary in terms of the type, frequency and severity across and within patients. The results provide adequate support for the content validity of the self- and caregiver-reported versions of the symptom-specific questionnaires. Minor modifications were made based on the feedback obtained. A

psychometric evaluation of the revised questionnaires is needed next to assess the construct validity, reliability, and responsiveness of the measures.

Patient preferences among third agent HIV medications: a US and German

perspective

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AIDS Care, 2008, 19(8)

ABSTRACT

The objective is to assess patient preferences for attributes associated with third agent HIV medications, including fosamprenavir/ritonavir (FPVr), fosamprenavir (FPV), lopinavir/ritonavir (LPVr), atazanavir (ATZ), and efavirenz (EFV). Subjects with HIV were recruited in the US and Germany to complete a computerized adaptive conjoint survey that assessed 13 attributes, including moderate to severe side effects, regimen convenience, drug resistance and efficacy. Literature on the target third-agent HIV drugs was used to identify percentage risk and severity level descriptions for each attribute. The derived preference (utility) weights for each attribute level informed the calculation of relative importance estimates for each attribute and the desirability of combinations of attributes matching the respective target third agents. The analysis included 288 HIV-positive participants (US: 132; Germany: 156), 205 of whom were treatment-experienced and 83 of whom were treatment-naïve. Of the 13 medication attributes evaluated, developing drug resistance, the risk of lipodystrophy, the risk of gastrointestinal side effects (diarrhea, nausea and vomiting) and regimen convenience had the greatest impact on preferences. The profile based on FPVr was most preferred. Differences in the risk of developing drug resistance, risk of lipodystrophy, risk of gastrointestinal side effects and regimen convenience would likely be most influential in the perceived relative value of a third-agent medication. Physicians may wish to consider these features, especially when discussing HIV treatment options with their patients.

Cross-cultural development and psychometric evaluation of a patient-reported health-related quality of life questionnaire for adults with haemophilia

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Haemophilia, 2008, 14(5)

ABSTRACT

Co-morbidities of hemophilia, such as arthropathy and blood-borne infections, can adversely affect the quality of life of adult patients with hemophilia. The purpose of this study was to develop and validate a hemophilia-specific health-related quality of life questionnaire for adults (HAEMO-QoL-A). Subjects with varying severities of hemophilia completed the HAEMO-QoL-A at baseline and 4 weeks. Other assessments included the SF-36 and Health Assessment Questionnaire - Functional Disability Index (HAQ-FDI). Two-hundred and twenty-one participants completed the 41-item HAEMO-QoL-A covering six domains (Physical Functioning, Role Functioning, Worry, Consequences of Bleeding, Emotional Impact and Treatment Concerns) and four independent items. Internal consistency was good-to-excellent (Cronbach's alpha-range: 0.75-0.95). Test-retest reproducibility was good, with intraclass correlation coefficients >0.80 except for the Emotional Impact domain (0.79).

Concurrent validity between the HAEMO-QoL-A total and subscale scores and all SF-36 subscale scores were generally good (correlations range: 0.13-0.87). Significant correlations between the HAEMO-QoL-A and the HAQ-FDI ranged from -0.14 to -0.69. There were non-significant correlations with the Treatment Concerns subscale and with the Worry subscale. The HAEMO-QoL-A discriminated significantly between adults with hemophilia by severity and HIV status. The Physical Functioning subscale discriminated between patients receiving prophylactic or on-demand therapy. The HAEMO-QoL-A is a valid and reliable instrument for assessing quality of life in hemophilia patients.

HIV patient insight on adhering to medication: a qualitative analysis

K M Beusterien E A Davis R Flood K Howard J Jordan

AIDS Care, 2008, 20(2)

ABSTRACT

Research on HIV medication adherence has relied mainly on quantitative methods. The objective of this study was to explore factors associated with adherence from the HIV-infected patient's perspective. Six focus groups were convened with treatment-experienced HIV-positive individuals. The discussions focused on issues that make it easy or difficult to adhere to HIV regimens. Thirty-five patients participated in the focus groups, which were conducted in Washington, D.C., and Los Angeles. The mean age was 48; 66% were male; 63% were black; and 40% contracted HIV through heterosexual contact. Six major themes emerged from the data that influenced adherence to medication: regimen complexity/medication features (including number of pills), lifestyle fit, emotional impacts (including worry, anger, stress and anxiety), side effects, medication effectiveness, and communication (including information from friends, physicians, and published sources). The data informed a conceptual framework, illustrating the possible interactions among these themes that can potentially be used by clinicians when discussing HIV treatment options with patients. This is potentially one of the first focus group studies concentrating on HIV medication adherence. The findings highlight specific factors that should be considered when trying to improve adherence and may be helpful in clinical decision-making.

Health state utilities for metastatic breast cancer

A Lloyd B Nafees J Narewska J Watkins

British Journal of Cancer, 2006, 95

ABSTRACT

The aim of the study was to obtain United Kingdom-based societal preferences for distinct stages of metastatic breast cancer (MBC) and six common toxicities. Health states were developed based on literature review, iterative cycles of interviews and a focus group with clinical experts. They described the burden of progressive, responding and stable disease on treatment; and also febrile neutropenia, stomatitis; diarrhoea/vomiting; fatigue; hand-foot syndrome (grade 3/4 toxicities) and hair loss. One hundred members of the general public rated them using standard gamble to determine health state utility. Data were analysed with a mixed model analysis. The study sample was a good match to the general public of England and Wales by demographics and current quality of life. Stable disease on treatment had a utility value of 0.72, with a corresponding gain of +0.07 following a treatment response and a decline by 0.27 for disease progression. Toxicities lead to declines in utility between 0.10

(diarrhoea/vomiting) and 0.15 (febrile neutropenia). This study underlines the value that society place on the avoidance of disease progression and severe side effects in MBC. This may be the largest preference study in breast cancer designed to survey a representative general public sample.

Evaluating preference weights for the Asthma Symptom Utility Index (ASUI) Across Countries

E Flood E De Cock A C Mork D A Revicki
Health and Quality of Life Outcomes, 2006, 4(51)

ABSTRACT

Background

The Asthma Symptom Utility Index (ASUI) is a preference-based outcome measure used in US clinical trials and cost-effectiveness studies for asthma. This study evaluated ASUI preference weights in Europe to determine whether the multi-attribute utility function, based on preferences from a US population, is generalizable across countries.

Methods

Data were collected from ninety asthma patients from Italy, France, and the United Kingdom using the Asthma Control Questionnaire, the Asthma Quality of Life Questionnaire, and the ASUI. Subjects rated their preferences for 10 asthma health states using a visual analogue scale (VAS) and a standard gamble (SG) interview.

Results

All multi-symptom states showed statistically significant differences ($p < 0.001$) between countries in mean VAS scores. Mean SG utility scores between the US and France and the US and Italy demonstrated statistically significant differences ($p < 0.001$) for three states: severe wheeze; moderate cough and wheeze; and moderate cough and dyspnea. Because of these differences, the multi-attribute utility functions derived within countries were somewhat different. Despite these differences, country-specific algorithms captured a similar rank ordering of patients by disease severity, were strongly correlated ($r = 0.971$ to 0.995), and demonstrated similar relationships with symptom and AQLQ scores.

Conclusion

Results of this study suggest that the ASUI may be a complementary patient-reported outcome for clinical studies and may be useful for applications in cost-effectiveness studies comparing different asthma treatments.

Validation Issues in Questionnaires for Diagnosis and Monitoring of Gastroesophageal Reflux Disease in Children.

L Kleinman D A Revicki E Flood
Current Gastroenterology Reports, 2006, 8(3)

ABSTRACT

Pediatric gastroesophageal reflux disease (GERD) is common in infants and children; diagnosis is often based on symptom presentation. This paper reviews psychometric characteristics and approaches to validation of currently available pediatric GERD questionnaires. Patient-reported outcomes allow disease and treatment to be characterized in meaningful ways to patients and clinicians. Outcome measures must demonstrate reliability and validity for use in practice and in clinical trials. Reliability assesses the consistency of measures, whereas validity examines whether the instrument measures what

it is purported to measure. Development of questionnaires for use with children also requires consideration regarding the respondent. Measures of pediatric GERD symptoms include the GERQ, GERQ-R, and GSQ. These measures have advantages and disadvantages with regard to feasibility, reliability, and validity. Questionnaires are lacking for children older than 4 years.

Validation of a patient-administered questionnaire to measure the activity impairment experienced by women with uncomplicated urinary tract infection: the Activity Impairment Assessment (AIA)

D Wild D Clayson K Keaing K Gondek
Health and Quality of Life Outcomes 2005, 3(42)

ABSTRACT

Background: To validate a questionnaire to assess the activity impairment associated with uncomplicated urinary tract infection (uUTI).

Methods: The Activity Impairment Assessment (AIA) assesses the amount of time an individual's work or regular activities have been impaired as a result of their UTI. The measure was completed by 276 women with uUTI who had participated in a prospective, open-label, non-comparative multi-centre clinical trial of CIPRO® XR (extended-release ciprofloxacin). Baseline scores on the King's Health Questionnaire (KHQ) and clinical symptom evaluations were collected for validation purposes.

Results: An exploratory factor analysis showed that all items loaded >0.84 on a single component. This uni-dimensional structure was supported by Rasch analysis. The AIA was found to have excellent levels of internal consistency (Cronbach's alpha = 0.93), convergent validity (all rs >.70) and divergent validity (rs = .078). The AIA displayed excellent discriminant validity in relation to clinical evaluations, and was found to be responsive to change across all clinical evaluations.

Conclusion: The uni-dimensional AIA shows high levels of internal reliability, convergent and divergent validity, discriminant validity and responsiveness. It is an excellent tool for measuring activity impairment in UTI.

Understanding patient preferences for HIV medications using adaptive conjoint analysis: feasibility assessment

K M Beusterien K Dziekan E Flood G Harding J Jordan
Value in Health, 2005, 8(4)

ABSTRACT

Objective: Choosing among HIV medications involve making trade-offs among various efficacy, convenience, resistance, and side-effect attributes. This study tested the feasibility of using adaptive conjoint analysis (ACA) to assess preferences (utilities) for HIV medication attributes.

Methods: HIV individuals were recruited through newspaper advertisements. Participants completed a computerized ACA survey that assessed 12 attributes, including side effects, regimen convenience, resistance, and efficacy. Literature on third-agent HIV drugs was used to identify percentage risk and severity level descriptions for each attribute. Based on the ACA-derived utilities, we assessed the relative importance of the attributes by averaging individually calculated importance and estimated the percentages that would prefer

selected HIV medications over others. To check validity of the ACA utilities, the survey also had respondents choose among medications with different attribute profiles.

Results: The 35 respondents were primarily African Americans (94%) and unemployed (54%). Of these, 28 (80%) provided consistent responses and were analyzed. Of the 12 medication attributes evaluated, the risk of developing resistance, regimen convenience, and the risk of sleep disturbance had the greatest impact on preferences; each accounting for more than 8.5% of the variation in preferences. These were followed by risk of drug failure (8.2%), cholesterol elevation (7.1%), diarrhea (7.1%) and nausea (6.9%). The ACA utilities accurately predicted patients' actual medication choices 75% of the time.

Conclusions: Adaptive conjoint analysis was successful in predicting HIV treatment preferences under different medication scenarios. Resistance, regimen convenience, and sleep disturbance would likely make the most difference in the perceived value of a third-agent HIV medication.

Self report quality of life measure for people with schizophrenia

G Wilson B Hesdon D Wild R Cookson C Farina V Sharma R Fitzpatrick C Jenkinson
British Journal of Psychiatry, 2000, 177

ABSTRACT

Background: Quality of life is the subject of growing interest and investigation.

Aims: To develop and validate a short self-report quality of life questionnaire (the Schizophrenia Quality of Life Scale SQLS)

Method: People with schizophrenia in Liverpool were recruited via the NHS. Items, generated from in-depth interviews, were developed into an 80-item self-report questionnaire. Data were factor analysed, and a shorter form measure was tested for reliability and validity. This measure was administered together with other self-report measures – SF – 36, GHQ – 12 and HADS – to assess validity.

Results: Data were analysed to produce a final 30-item questionnaire, comprising three scales ('psychosocial', 'motivation and energy', and 'symptoms and side-effects') addressing different SQLS dimensions. Internal consistency reliability of the scale was found to be satisfactory. There was a high level of association with relevant SF-36, GHQ-12 and HADS scores.

Conclusions: THE SQLS was completed within 5-10 minutes. It possesses internal reliability and construct validity, and promises to be a useful tool for the evaluation of new treatment regimes for people with schizophrenia.

The level of haemoglobin in anaemic cancer patients correlates positively with quality of life

M Lind C Vernon D Cruickshank P Wilkinson T Littlewood N Stuart C Jenkinson H Doll D Wild
British Journal of Cancer, 2002, 86

ABSTRACT

The aim of this study was to assess the relationship between haemoglobin level and quality-of-life in anaemic cancer patients.

Patients, diagnosed with one of four cancers, were recruited if their haemoglobin level was 512 g dl⁻¹ (female) or 513 g dl⁻¹ (male). The condition-specific Functional Assessment of Cancer Therapy – Anaemia and the generic SF-36 were used to assess quality-of-life. Thirty-

six per cent of the 179 recruited patients had breast cancer, 28% ovarian cancer, 25% lung cancer, and 11% multiple myeloma. Their mean (s.d.) haemoglobin level was 10.66 (1.04) g dl⁻¹. Partial correlations controlling for the potentially confounding effects of age, gender, and time since diagnosis found significant positive relationships between haemoglobin and all domains of the Functional Assessment of Cancer Therapy – Anaemia, and with all but two of the SF-36 domains. On linear regression controlling for the same factors, each unit haemoglobin rise equalled an average 8.19 Functional Assessment of Cancer Therapy – Anaemia, and an average 6.88 Functional Assessment of Cancer Therapy – Fatigue, increase. Haemoglobin accounted for a similar amount of variability (8%) in SF-36 scores. In conclusion, quality-of-life has been found to be significantly positively related to haemoglobin level in anaemic cancer patients. This suggests that normalisation of haemoglobin in cancer patients is likely to increase their quality-of-life. The greater sensitivity of the condition-specific Functional Assessment of Cancer Therapy – Anaemia compared with the generic SF-36 suggests that the Functional Assessment of Cancer Therapy – Anaemia can be used alone to assess quality-of life in this patient group.