All patients are entitled to equal access to health care resources. The Department of PharmacoEconomics at University of Texas MD Anderson Cancer Center (UTMDACC) administers a Patient Assistance Program (PAP) that provides assistance to indigent patients with free pharmaceuticals for their therapy. Drug cost is recovered through a drug reimbursement program offered by pharmaceutical companies. OBJECTIVE: The objective of this report is to examine trends in drug cost savings to indigent patients at UTMDACC using the PAP model. METHODS: A retrospective study using data from September 1996 to August 2000 was conducted to determine the value of the program. Patients were enrolled in this program if they qualified based on the Financial Classification Scale. Uninsured patients as well as under-insured patients were considered in this study. Data was analyzed to evaluate the trend in cost savings for the three fiscal years. RESULTS: Over $334 million was spent on drug cost over the period. There was an average increase of 22% per year in drug cost. The indigent patients accounted for 9% of the total patient population at UTMDACC. An estimated $33 million was spent on drugs for indigent patients during that period. The PAP system recovered a total of $16.8 million; $4.1M (1997), $4.3M (1998), $3.5M (1999), and $4.9M (2000). This accounts for 51% in drug cost recovery through this program. The fluctuation in cost saving was attributed to changes in the number of programs, number of patients enrolled, and product mix. CONCLUSION: The PAP system has provided free drugs to patients without financial resources and reduced the economic burden of this population on the health care institution. The program has created goodwill between the pharmaceutical companies, the health care institution, the patients and the community.

NO SPECIFIC OR MULTIPLE DISEASES-QUALITY OF LIFE & PREFERENCE-BASED MEASURES

LESSONS LEARNED FROM DEVELOPING A PSYCHOMETRICALLY BASED SEDATION QUESTIONNAIRE IN PHARMACOLOGICALLY PARALYZED CRITICALLY ILL PATIENTS

Kane SL, Dasta JF, Pathak DS
The Ohio State University, Columbus, OH, USA

OBJECTIVE: To share lessons learned from developing a reliable and valid questionnaire for adequacy of sedation in pharmacologically paralyzed critically ill patients. METHODS: In phase 1, seven experts listed 21 characteristics describing anxiety in pharmacologically paralyzed patients. In phase 2, two scenarios were created illustrating the experience of paralysis: one with and one without receiving a sedative. A convenience sample of 30 people evaluated scenarios to determine the importance of characteristics obtained from phase 1 using a five-point scale. Items were reduced to the 10 most important characteristics (mean ≥3). Based on these results, the final instrument consisted of 12 questions: 2 categorical addressing memory of the experience and 10 referred to characteristics of anxiety. In phase 3, two groups of critically ill patients were administered the questionnaire: 1) sedated only and 2) sedated and pharmacologically paralyzed. The questionnaire was administered twice for reliability. Questionnaire results were compared to subjective and objective sedation monitoring tools for validity. Calculated sample size was 20 for each group. RESULTS: During six months, 21 patients consented to participate. Twelve patients died and nine patients (6 sedated, 3 sedated/paralyzed) were administered questionnaires. Five patients (3 sedated, 2 sedated/paralyzed) did not remember the intensive care unit experience. Two of three patients in the sedated group who answered the questionnaire found it difficult to remember over time. One sedated/paralyzed patient who answered the questionnaire received a sedative without amnesic properties and felt anxious during therapy. The distressful feeling of this patient was comparable to findings of the objective sedation tool. CONCLUSIONS: Mortality in this critically ill patient population was high. Due to amnestic properties of sedatives most patients did not remember the experience. Of patients who remembered, their memory deteriorated over time. Based on lessons learned, it may require 2–3 years to achieve the necessary sample size.

ROLE OF HEALTH RELATED QUALITY OF LIFE OUTCOMES IN THE EUROPEAN DRUG REGULATORY PROCESS: A REVIEW OF THE EMEA DOCUMENTS

Acquadro C, Staniek V, for the ERIQA Group
Mapi Research Institute, Lyon, France

INTRODUCTION: The ERIQA Group aims to create guidance for European regulatory authorities on the assessment of the quality of HRQL studies in clinical trials and to evaluate the validity of HRQL claims. OBJECTIVES: To identify disease or drugs in which a formal HRQL assessment is recommended. To identify measures and methods recommended. To evaluate the reliability of recommendations across documents. METHODS: Information was searched on the EMEA website (www.eudra.org/emea.html). A research was performed with the two key words: “Quality of life” and “QoL” All the documents retrieved were reviewed. RESULTS: 133 documents were retrieved excluding duplicates (129: Quality of Life, 25: QoL). 19 documents derived from the Efficacy Working Parties (EWP) including nine notes for guidance (Weight Control, Cancer, Chronic Peripheral Arterial Occlusive Disease, Cardiac Failure, Stable Angina Pectoris, Antarrhythmic, Parkinson, Alzheimer, Multiple Sclerosis), concept paper (3), points to consider (5) and position statements (2). Only one document was a note for guidance for ICH. 104 European Public Assessment Report (EPAR) were retrieved, representing 26 products. Nine miscellaneous documents were found in-
OBJECTIVE: This paper reviews current approaches to defining clinically meaningful change in health-related quality of life (HRQOL). METHODS: Definitions of clinically meaningful change are discussed. Psychometric properties of HRQOL instruments necessary for identifying clinically meaningful change are identified. Two broad methods for identifying clinically meaningful change are contrasted: anchor-based methods and distribution-based methods. Anchor-based methods include forced-choice paradigms, global change ratings, receiver operating characteristic techniques, goal attainment scaling and external event methods. Distribution-based methods include individual effect size, the Guyatt responsiveness index, the Jacobson-Traux reliable-change index (and subsequent variations), standard error of measurement, and hierarchical linear modeling. Strategies for validating clinically meaningful change measures are discussed. RESULTS: Anchor-based and distribution-based methods have both advantages and limitations, and neither appears superior to the other. Anchor-based methods provide a source for external validation, but are dependent on the specific anchors being used. Distribution-based methods provide a statistical basis for decision-making, but may vary on the basis of sample characteristics. CONCLUSIONS: The use of multiple methods to define clinically meaningful change is strongly recommended. Factors to consider in defining clinically meaningful change include the severity of the baseline value, the direction of change, and the importance of the change to the individual.

DEFINING CLINICALLY MEANINGFUL CHANGE IN HEALTH-RELATED QUALITY OF LIFE
Crosby RD, Kolotkin RL, Williams GR
1Neuropsychiatric Research Institute, Fargo, ND, USA; 2Duke Medical Center, Durham, NC, USA; 3Knoll Pharmaceuticals, Mount Olive, NJ, USA

OBJECTIVES: This paper reviews current approaches to defining clinically meaningful change in health-related quality of life (HRQOL). METHODS: Definitions of clinically meaningful change are discussed. Psychometric properties of HRQOL instruments necessary for identifying clinically meaningful change are identified. Two broad methods for identifying clinically meaningful change are contrasted: anchor-based methods and distribution-based methods. Anchor-based methods include forced-choice paradigms, global change ratings, receiver operating characteristic techniques, goal attainment scaling and external event methods. Distribution-based methods include individual effect size, the Guyatt responsiveness index, the Jacobson-Traux reliable-change index (and subsequent variations), standard error of measurement, and hierarchical linear modeling. Strategies for validating clinically meaningful change measures are discussed. RESULTS: Anchor-based and distribution-based methods have both advantages and limitations, and neither appears superior to the other. Anchor-based methods provide a source for external validation, but are dependent on the specific anchors being used. Distribution-based methods provide a statistical basis for decision-making, but may vary on the basis of sample characteristics. CONCLUSIONS: The use of multiple methods to define clinically meaningful change is strongly recommended. Factors to consider in defining clinically meaningful change include the severity of the baseline value, the direction of change, and the importance of the change to the individual.

CROSS-CULTURAL VARIATIONS IN SF-12 SCORES AMONG INDIVIDUALS WITH VARIOUS HEALTH CONDITIONS
Bolge SC, Eschmann B, Donohue JA
Consumer Health Sciences, Princeton, NJ, USA

OBJECTIVE: To examine the cross-cultural differences of health-related quality of life among people with various health conditions across four countries. METHODS: Analyses were based on 12-page questionnaires mailed to adults in 2000. A total of 38,677 responses were received from France, Germany, Great Britain, and the US Results were subsequently weighted and projected to the national populations of these four countries. Weighting was based on gender, age, and region for the European countries and gender, age, race, and region for the US Participants reported whether they were diagnosed with the following: arthritis, asthma, depression, diabetes, GERD, high blood pressure, high cholesterol, migraines, nasal allergies, or osteoporosis. Physical and mental health status were defined by summary measures of the SF-12 scale. RESULTS: The French reported lower mental health status but better physical health status than people in Germany, Great Britain, and the US, regardless of diagnosed health conditions. The opposite was found among arthritis, asthma, and GERD sufferers in Germany, who reported the best mental health status but the lowest physical health. Respondents from Great Britain and the US generally reported SF-12 scores that fell between those of France and Germany, with three notable exceptions. Among people diagnosed with depression, those in the US reported better mental health status. Among people diagnosed with high blood pressure or high cholesterol, those in Great Britain reported the lowest physical health status. CONCLUSION: Self-reported quality of life varied by country regardless of condition. Researchers should consider cross-cultural variations in self-reported quality of life measures when conducting multinational trials. Collapsing data could obscure effects.

TRANSLATING SF-36 SCORES INTO PREFERENCES: AN EXAMINATION OF THE PERFORMANCE OF TWO PREDICTIVE EQUATIONS
Meletiche DM, Roberts CS, Lofland JH
Thomas Jefferson University, Philadelphia, PA, USA

Despite widespread use of the SF-36, its use in cost-utility analyses has been precluded by its inability to measure patient preferences. To overcome this obstacle, various investigators have derived equations to estimate preference scores from the SF-36. OBJECTIVE: To compare two methods of estimating preference values from SF-36 scores. METHODS: A convenience sample of patients completed the SF-36 and EuroQol during their initial visit to a specialty headache center. Preference scores were estimated from the SF-36 using two equations, one developed by Fryback and the other by Brazier. The performance of each equation was assessed by calculating the correlation coefficient between the estimates and actual preference scores from the EuroQol. Mean preference scores from each method were compared using one-way repeated measures ANOVA. RESULTS: Forty-seven