lactic treatment of hemophilia. Patients and caregivers completed questionnaires and provided verbal feedback addressing several open-ended questions, including type and duration of bleeds, disability, impact of treatments on lifestyle and their perceptions about standard utility. RESULTS: Twenty-one patients or caregivers of children with bleeding disorders participated. Important implications for the model were that a) Patients reported that muscle bleeds can be more painful and last longer (i.e., associated with lower utility) than joint bleeds, which are generally considered more disabling; b) Patients reported that the course (utility, improvement) of a typical bleed differs by the type of joint (e.g., muscles), not necessarily by bleed severity alone; c) Although patients reported a loss of utility during a bleed, they gradually regained the same level of utility/function, hence disability was not perceived as a linear process; d) Adult patients reported that nowadays, unlike them, children with hemophilia receiving prophylactic treatment are able to play sports (e.g., ice hockey), thereby having a good quality of life, an aspect not captured by standard utility instruments; e) Although caregiver-administered instruments for assessing quality of life in children with hemophilia are available, caregivers noticed concern about accurately rating their child’s health status based on their experience. CONCLUSIONS: Although qualitative, patients’ perspectives revealed in this focus group demonstrated important utility consequences for the economic model that are not represented in the literature.

PSY60 LARGE-SCALE, PROSPECTIVE, OBSERVATIONAL STUDIES IN PATIENTS WITH PSORIASIS AND PSORIATIC ARTHRITIS: A SYSTEMATIC AND CRITICAL REVIEW.

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BACKGROUND: Observational studies, if conducted appropriately, play an important role in the decision-making process providing invaluable information on effectiveness, patient-reported outcomes and costs in a real-world environment. OBJECTIVES: A systematic review of large-scale, prospective, cohort studies with the aim of (a) summarising design characteristics, the interventions or aspects of the disease studied and the outcomes measured and (b) investigating methodological quality. METHODS: We included prospective, cohort studies which included at least 100 adult patients with psoriasis or psoriatic arthritis. Studies were identified through searches in electronic databases (Pubmed, Medline, Cochrane library, Centre for Reviews and Dissemination). Information on study characteristics were extracted and tabulated and quality assessment of the papers covering 16 cohorts met the inclusion criteria. There were 10 treatment-related studies, only 2 of which provided a comparison between treatments, and 6 non-treatment studies which examined a number of characteristics of the disease including mortality, morbidity, cost of illness and health-related quality of life. All studies included a clinical outcome measure and 11 included patient-reported outcomes, however only two studies reported information on patient utilities and two on costs. Quality ranged from 41% to 89%. Studies did well on a number of quality assessment questions including having clear objectives, documenting selection criteria, providing a representative sample, defining interventions/characteristics under study, defining and using appropriate outcomes, describing results clearly and using appropriate statistical tests. The quality assessment criteria least adhered to involved questions regarding sample size calculations, describing potential selection bias, defining and adjusting for confounders and losses to follow-up, and defining and describing a comparison group. CONCLUSIONS: The review highlights the need for well designed prospective observational studies on the effectiveness, patient-reported outcomes and economic impact of treatment regimes for patients with psoriasis or psoriatic arthritis in a real-world environment.

HEALTH CARE DECISION-MAKER’S CASE STUDY POSTER SESSION

PCASE1 UTILIZING EVIDENCE FROM DIFFERENT LEVELS IN THE REIMBURSEMENT PROCESS OF NEW MEDICAL TECHNOLOGIES—ADVANCED REINAL CELL CARCINOMA FIRST LINE THERAPY IN POLAND 2008–2009

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ORGANIZATION: Agency for Health Technology Assessment in Poland (AHTAPol) serves as an advisory body to support the Minister of Health and relevant policy makers in the decision-making process in the public sector of health care in a 38 million people country. PROBLEM OR ISSUE ADDRESSED: During 2007 in Poland, patients with metastatic renal cell carcinoma (mRCC), seventh-leading cause of death in men and in ninth-leading cause in women due to cancer, had access only to immunomodulatory therapy. GOALS: Necessity of providing access to cost-effective new treatments in the first line of therapy of mRCC. OUTCOMES ITEMS USED IN THE DECISION: Clinical efficacy, safety, evidence from RCT (RCT interim analysis, patient registry), quality of life, cost-effectiveness/cost-utility. IMPLEMENTATION STRATEGY: Consultative Council (CC) of AHTAPol debated on the subject multiple times and gave a new decision after each manufacturer’s submission concerning a new HTA report updated as soon as the new information was published. RESULTS: In 2008–2009, CC debated over 3 claims for the reimbursement of sunitinib and one for the reimbursement of bevacizumab in the indication: metastatic renal cell carcinoma (mRCC), seventh-leading cause of death in men and in ninth-leading cause in women due to cancer, had access only to immunomodulatory therapy. RESULTS: was published on the subject multiple times and given a new decision after each manufacturer’s submission. IMPLEMENTATION STRATEGY: On August, 6, 2008, CC rejected submission of bevacizumab on the grounds of an acceptably low level of submitted HTA report, especially in the sections comprising safety issues and economical analysis. Third and final submission of sunitinib was submitted to CC on February 2, 2009. CC was convinced of the evidence from two previous submissions and a newly published data of BC Cancer Registry—a registry of patients with mRCC. This data on practical effectiveness showing prolongation of OS, rather than unreliable evidence on efficacy derived from conference materials, persuaded CC to recommend reimbursement of sunitinib for Polish patients. After December 12, 2009 CC rejected second submission of bevacizumab because there was no evidence of its superiority over already reimbursed sunitinib. LESSONS LEARNED: Utilising evidence from different levels can broaden decision maker’s perspective and justify coverage of sometimes expensive but socially demanded new treatments. In certain cases, there is a lack of evidence from the irrefutably well designed and conducted clinical controlled trials and thorough analysis of observational studies can give enough additional data to issue an informed decision. Data from large patient registries can be crucial to the decision-making process.

PCASE2 DEVELOPING CRITERIA FOR USE FOR CONTINUOUS GLUCOSE MONITORING (CGM) SENSORS IN A VETERAN POPULATION

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ORGANIZATION: Veteran Affairs San Diego Healthcare System (VADHS) is a 242-bed medical facility that provides comprehensive medical, surgical, mental health, rehabilitation extending beyond the VA facilities, and obstetric services. VADHS is part of the Department of Veterans Affairs which is an integrated health care organization that operates using a national formulary, provides outpatient and inpatient specialty services and uses a comprehensive electronic medical record system. PROBLEM OR ISSUE ADDRESSED: The absence of an accepted system for evaluating appropriate utilization of disposable medical supplies present decision makers with concern for how resources are managed in this category of budget expenditures. There is no current standard in place for technology assessments for durable medical equipment (DME) requiring disposable medical supplies in a pharmacy benefit management (PBM) plan with the potential for extensive budgetary and utilization management challenges to our health care system. With the introduction of CGM to the US market, the American Diabetes Association has recognized CGM as a useful tool for clinicians and patients, describing systems with potential benefits including: CGM sensors provide information on glucose, and can be a gap in communication that was identified within the CGM device approval process. RESULTS: The absence of an accepted system for evaluating appropriate utilization of disposable medical supplies present decision makers with concern for how resources are managed in this category of budget expenditures. There is no current standard in place for technology assessments for durable medical equipment (DME) requiring disposable medical supplies in a pharmacy benefit management (PBM) plan with the potential for extensive budgetary and utilization management challenges to our health care system. With the introduction of CGM to the US market, the American Diabetes Association has recognized CGM as a useful tool for clinicians and patients, describing systems with potential benefits including: CGM sensors provide information on glucose, and can be implemented as devices for measuring glucose levels by alerting patients of the potential danger prior to a hypoglycemic event. Annualized cost for CGM devices and sensors ranged from $740 to $3520 per patient based on patients refill rates in FY2009. $18,480. Annualized cost for CGM device and sensors ranged from $740 to $3520 per patient based on patients refill rates in FY2009.

LESSONS LEARNED: Utilizing evidence from different levels can broaden decision maker’s perspective and justify coverage of sometimes expensive but socially demanded new treatments. In certain cases, there is a lack of evidence from the irrefutably well designed and conducted clinical controlled trials and thorough analysis of observational studies can give enough additional data to issue an informed decision. Data from large patient registries can be crucial to the decision-making process.

Abstracts

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Interim analysis of an international multicenter III phase randomized controlled trial (RCT) and 4 observational studies. The claim was rejected, on April 1, 2008, on the grounds of insufficient data on overall survival (OS) and unacceptable level of cost-effectiveness of app. USD 330 thousand per LYG, much higher than three-fold GDP recommended by WHO. The second and third submissions for the model were submitted together with final results of the III phase trial available as conference presentation and abstract, was also rejected by CC on October 28, 2008. Claiming that a conference presentation and abstract are not sufficiently reliable evidence, CC concludes, muscle, no nociception was presented and cost-effectiveness remained unacceptable high. Meanwhile, August 6, 2008, CC rejected submission of bevacizumab on the grounds of an acceptably low level of submitted HTA report, especially in the sections comprising safety issues and economical analysis. Third and final submission of sunitinib was submitted to CC on February 2, 2009. CC was convinced of the evidence from two previous submissions and a newly published data of BC Cancer Registry—a registry of patients with mRCC. This data on practical effectiveness showing prolongation of OS, rather than unreliable evidence on efficacy derived from conference materials, persuaded CC to recommend reimbursement of sunitinib for Polish patients. After December 12, 2009 CC rejected second submission of bevacizumab because there was no evidence of its superiority over already reimbursed sunitinib. LESSONS LEARNED: Utilizing evidence from different levels can broaden decision maker’s perspective and justify coverage of sometimes expensive but socially demanded new treatments. In certain cases, there is a lack of evidence from the irrefutably well designed and conducted clinical controlled trials and thorough analysis of observational studies can give enough additional data to issue an informed decision. Data from large patient registries can be crucial to the decision-making process.