PODIOUM SESSION IV: RISK ASSESSMENT STUDIES

RA1
CAN THE PUBLIC’S RISK PREFERENCE WHEN WEIGHING A DRUG’S RISK/BENEFIT BE TRUSTED?
University of California, San Francisco, San Francisco, CA, USA

OBJECTIVES: To measure utility (risk preference) of individuals deciding to take a multiple sclerosis (MS) drug given varying risks/benefits. METHODS: A computerized measure based on PosSEQFT software was developed to measure Standard Gamble utility. 45 healthy individuals were presented with 9 health states varying MS disease severity (mild, moderate, severe) and asked their preferences to remain in that state or gamble on taking a drug with varying probabilities of 3 levels of MS improvements (mild, average, substantial) but also corresponding probabilities of death. Student’s t-test analyzed affected utility, gender, age, education, and MS experience on utility.

RESULTS: Subjects ranged from 16–69 years (mean = 40 years) and 44.4% knew someone with MS. In general, healthy individuals were willing to accept substantial risk of death for even mild MS improvements and accepted more risk for severe MS and greater disease improvements. The lowest mean score was for mild MS improvement given mild MS (0.082; 95% CI [0.045-0.120]). The highest score was for substantial MS improvement given severe MS (0.382; 95% CI [0.295-0.646]). Only education significantly affected utility for mild improvement in mild MS (p = 0.03). Individuals with bachelor’s degree or less had 7.7% higher score than post-graduates.

CONCLUSIONS: We found it surprising how much risk of death healthy individuals were willing to accept (8–38%). This is much greater than the 1/1000 actual risk for natalizumab in MS treatment, which the FDA removed from the market. MS patients voiced preferences helped get that drug back on the market, showing importance of shared health decision-making. The FDA and physicians might find it difficult to approve or prescribe drugs with 38% risk of death. However, these preferences indicate healthy individuals may be less risk-averse than the FDA or physicians, emphasizing the importance of including the public when weighing risks and benefits of drugs.

RA2
RISK OF DIABETES ASSOCIATED WITH THE USE OF ATYPICAL ANTIpsychotics in children and adolescents – a Texas medicaid study
Nagar SP1, Mhita S2, Zwedl P1, Chen J1
1University of Houston, Houston, TX, USA

OBJECTIVES: To examine the risk of developing diabetes among children and adolescents who received atypical antipsychotic drug. MEIHDS: This study is a retrospective cohort analysis using 2003–2004 Texas Medicaid claims data. Patients were included in the cohort if they met the following criteria: 6 to 18 years of age, new users of atypical antipsychotics and had not received a diagnosis of diabetes (ICD-9-CM code: 250.xx) or a prescription claim of an anti-diabetic drug both before and one month after the initiation of an antipsychotic treatment. Risk of newly developed diabetes following antipsychotic treatment was observed during a 12 month follow-up period. Kaplan-Meier survival curves and Cox proportional-hazards regression model were employed to examine the time dependent risk (hazard) of diabetes and hazards ratio were obtained. Age, gender, race and exposure to other diabetogenic medications were controlled in the analysis. RESULTS: A total of 40% patients were identified as new atypical antipsychotic users. The average age of the cohort was 11 years, most of the patients were male (65%), and Hispanics (36%). Cox-proportional hazard model analysis revealed no significant association between use of atypical antipsychotic and risk of diabetes (Hazards Ratio, HR = 1.11, 95% CI = 0.81–1.53). Children in the older age group of 15–18 years, females and Hispanics had higher risk of developing diabetes. Concurrent treatments with corticosteroids, beta-blockers, thiazides and antiprostaglandins were also associated with increased incidence of diabetes.

CONCLUSIONS: Our results indicate that there is no increase in the risk of developing diabetes in pediatric patients receiving atypical antipsychotics. The discrepancy in the findings between adults and children implies that psychotropic medications might function differently in pediatric population from adults. It is necessary to comprehensively assess the risks associated with common off-label medications, such atypical antipsychotics, in children and adolescents.

RA3
A BAYESIAN DECISION-ANALYTIC ECONOMIC MODEL TO OPTIMIZE ALLOCATION OF RISK IN PAY-FOR-PERFORMANCE PAYMENT ARRANGEMENTS
Mulick B1, Hoffenbaek C2
1Risk: Sharing Solutions, Collegeville, PA, USA, 2Penn State College of Medicine, Hershey, PA, USA

OBJECTIVES: Performance-based payment arrangements for innovative drugs seek to allocate financial risk of interventions between manufacturers and payers. Given operational challenges in monitoring real-world outcomes, effective risk sharing mechanisms may require prediction of incremental survival/utility-adjusted life years (QALYs) conditional on surrogate markers, for example, complete remission (CR) or partial remission (PR). In accepting a risk-sharing arrangement that reimburses only for remitters, payers should minimize the “false-positive” (FP) risk: early remitters who subsequently have limited QALYs. Manufacturers should minimize the “false-negative” (FN) risk: early non-remitters who subsequently have prolonged QALYs.

A Bayesian decision framework can be used to choose among multiple likelihood (predictive) priors to optimize the posterior economic risk trade-off for payers and manufacturers. METHODS: A Bayesian decision-analytic, hypothetical data-based, cost-effectiveness model was developed. Prior probabilities and QALYs were assigned for 6-, 1-, and 1-month survival, and treatment costs. A plausible prior likelihood (predictive) structure represented the (ROC) relationship between the specificity and sensitivity of CR/PR in predicting survival. Expected (posterior) probabilities of survival, conditional on CR/PR, were generated. At a threshold of $30,000/QALY, the cost-effectiveness of the intervention, conditional on achieving CR/PR, and an optimal sensitivity-specificity trade-off was derived. RESULTS: At a hypothetical treatment cost of $5,000/month for a 4-month cycle, a minimal FP of 13% (maximum specificity of 87%) and a minimal FN of 33% (maximum sensitivity of 67%) emerged as necessary conditions to be accepted by payers and manufacturers respectively to ensure viable risk-sharing. At higher sensitivity, payer risk did not meet the reimbursement threshold, while at higher specificity, manufacturers would assume excessive financial risk. Other illustrations will be discussed. CONCLUSIONS: Manufacturers should propose evidence-based payment arrangements that utilize clinical trial data to develop economic implications of being at various points on the ROC curve in order to optimize the trade-offs between payer and manufacturer incentives.

PODIOUM SESSION IV: RESPIRATORY-RELATED DISORDERS – Outcomes Research & Health Care Policy Studies

RR1
QUARTERLY ASSESSMENT OF SHORT ACTING β-AGONIST USE AS A PREDICTOR OF SUBSEQUENT HEALTH CARE SERVICES USE FOR ASTHMA IN THE U.S.
Blanchette CT1, Silver H1, Petersen H2, Kamble V1, Hodges D2, Gutierrez B2
1Loveland Respiratory Research Institute, Albuquerque, NM, USA, 2University of North Carolina at Charlotte, Charlotte, NC, USA, 3AstraZeneca, Wilmington, DE, USA

OBJECTIVES: Previous research has shown that high utilization of short-acting β-agonsists (SABA) is associated with asthma exacerbations, however annual assessments present significant barriers to real-time assessment needed for efficient care. We explored a quarterly assessment of high utilization SABA utilization patterns on subsequent asthma exacerbations. MEIHDS: A retrospective cohort study using conducted PHARMetrics database included health plan members (aged 6–66 years; n = 39,604) with asthma and two-year continuous enrollment between 7/2003–6/2007, >1 hospitalization/emergency department (ED) or 2 outpatient claims of International Classification of Diseases, 9th Revision, Clinical Modification (ICD-9-CM) code 493.XX and ≥1 asthma medication claim in each of the two years of observation. Patients with COPD and diseases associated with chronic oral steroid use were excluded. SABA utilization was converted to canister-count in their first observed quarter and categorized into 0, 1, 1–2, 3 or >3. Risk of an asthma exacerbation in the subsequent quarter, defined as an oral steroid claim, hospitalization, or ED visit, was assessed using logistic regression, controlling for age, sex, race, smoking status, and overall first quarter outcomes. RESULTS: The cohort included 33,951 patients aged 6–67 (36%) and 59,563 aged 18–66 (64%) of which 64% had ≥1 SABA canisters, 18% had ≥1, 9% had ≥2, 9% had ≥3. Compared to 0 canisters, higher SABA utilization was associated with higher exacerbation risk (HR: 1 [OR:1.0]), C.I. 0.92–1.12), 1–2

Abstracts

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