APPLICATION OF COST-EFFECTIVENESS LOGIC TO US MANAGED CARE DRUG FORMULARIES: LONG-TERM OUTCOMES OF A VALUE-BASED FORMULARY

OBJECTIVES: Cost-effectiveness analysis (CEA) is explicitly used for informing drug coverage decisions in many countries but not in the United States. Evidence suggests that failure to incorporate value considerations in drug coverage decisions may lead to reduced economic efficiency in the form of increased costs or worsened health outcomes. Yet the use of CEA in the context of binary coverage decisions (yes or no) may not be politically or socially feasible in the US. In 2010, Frenema Blue Cross implemented a value-based formulary (VBF) that uses CEA to determine the treatment level—not binary coverage—for each drug in the formulary; drugs with lower incremental cost-effectiveness ratios (ICERs) are assigned lower copayments, drugs with higher ICERs are assigned higher copayments. The objectives of this study were to assess the impact of Frenema’s VBF on healthcare costs and outcomes. METHODS: We utilized an interrupted time series design with concurrent control group in order to examine the impact of the VBF on both pharmacy and medical costs for enrollees and the Medicare plan separately and to examine the impact of the VBF on both emergency department visits and acute hospitalizations. In order to accomplish these aims, we utilized segmented regression models with two-part generalized estimating equations for analysis. RESULTS: Preliminary descriptive analysis suggests that over the 4 years of observation, comparing the period before VBF implementation to the period after VBF implementation, both medical and pharmacy costs increased more in the control group ($38 37 and $4.79 per member per month (PMPM)) than in the VBF group ($36 17 and $4.58 PMPM). The number of emergency department visits and acute hospitalizations did not change in either group. CONCLUSIONS: Preliminary analyses suggest that the use of cost-effectiveness principles in the US context may lead to greater economic efficiency. Subsequent analyses utilizing greater control for confounding will establish more valid estimates of outcomes and costs.

ANALYSIS OF NICE DRUG TECHNOLOGY APPRAISALS (2001-SEPTEMBER 2014)

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OBJECTIVES: This study assessed the NICE Drug Technology Appraisals published in the period 2001-September 2014. METHODS: The list of NICE guidance, including published guidance, in development and consultations was extracted from NICE webpage. Descriptive statistics and chi-square were used in the analysis. RESULTS: In September 2014, NICE listed 594 guidance documents, including 246 technology appraisals (TAs), of which 218 were drug TAs. Of these, 128 were for different drugs, combinations, or drug classes. 75.8% of the drug TA evaluated was recommended by NICE in the National Health Service (NHS), however 17.0% of them were not recommended. NICE was unable to recommend them because no evidence submission was received from the drug sponsor in 7.2% of the TA. In 46.2% of the 91 TA published in 2010-2014 recommended by NICE, the sponsor agreed a patient access agreement with the Department of Health to provide a confidential discount. The percent-age of drug TAs recommended by NICE increased over time from 89.5% (n=49) in 2001-2004, to 71.7% (n=91) in 2005-2009, and 75.8% in 2010-2014 (p<0.001). There were six therapeutic classes with 10 or more TAs recommended by NICE: Anti-diabetic medications (37, 77.8%), cardiovascular conditions (20, 95.0%), musculoskeletal conditions (16, 81.3%), infections (11, 90.9%), and neurological conditions (10, 90.0%). CONCLUSIONS: Most of the TA resulted in a positive recommendation by NICE for using the drug in the NHS. Oncology and blood and immune system condition had the lowest percentage of TA resulting in a positive evaluation from NICE. Over 45% of the TA published after 2010 resulted in a confidential discount provided by the sponsor company to the NHS.

IQWIG EARLY BENEFIT ASSESSMENTS OF TYPE 2 DIABETES THERAPIES

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OBJECTIVES: Since 2011, the Institute for Quality and Efficiency in Health Care (IQWiG) has awarded very few anti-diabetic therapies an "added benefit" over current standard therapies status. IQWIG dossier evaluations were examined to determine if new therapies do not demonstrate "added benefit" or if results may be due to a lack of comparative evidence. METHODS: Results from IQWIG dossier assessments for all anti-diabetic drug therapies requiring early benefit assessment were examined. IQWIG’s website was searched for "diabetes," identifying Albiglutide, Canagliflozin, Dapagliflozin, Empagliflozin, Linagliptin, Lixisenatide, Saxagliptin, and Vildagliptin. Dossier assessment results were reviewed to all sub-indications to determine if added benefit had been evaluated. RESULTS: In total, 14 dossier assessments (9 monotherapies; 5 combination therapies) were reviewed representing 48 sub-indications. Of the 48 sub-indications, 2 were designated as having "added benefit" or "hint of added benefit," the remaining 46 did not. The most common reasons for no evidence of "added benefit" were: no evidence submitted (n=26), lack of power to demonstrate added benefit (n=12), non-compliance with approved dosages (n=12), and non-compliance with approved dosages not tailored to patient (n=6), study duration too short (n=5). CONCLUSIONS: In most cases, the evidence needed to perform an IQWIG early benefit assessment for drug therapies for Type 2 Diabetes did not exist. Moreover, studies with the potential to provide the appropriate evidence lacked use of approved dosage, indicated popula-tions, or comparable study arms preventing a complete assessment of whether such indiciations of an anti-diabetic therapy provided an "added benefit" to patients. Future clinical development plans should include well-designed comparative stud-ies to improve likelihood of reimbursement and patient access.

PATIENT PREFERENCE STUDIES

PATIENTS’ AND PHYSICIANS’ TIME TRADE-OFF PREFERENCES FOR ADVERSE OUTCOMES ASSOCIATED WITH METASTATIC COLORECTAL CANCER TREATMENTS

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OBJECTIVES: To elicit health-state utilities for adverse outcomes associated with metastatic colorectal cancer. METHODS: Patients and physicians completed time-trade-off (TTO) questions. Health states were drafted and refined based on literature review, and patient and clinician interviews. Four adverse conditions were considered: severe rash (rash), severe bloody diarrhea, and gastrointestinal perforations. Respondents evaluated the risk of serious bleeding, heart attack, and gastrointestinal perforations. Three event risk levels were randomized across events and respondents. Rash was presented as a deterministic outcome, so respondents evaluated the impact of experiencing the rash, not as the risk of developing rash. Patients and physicians evaluated the health states in TTO questions that provided a range of time in the adverse health state that would leave respondents indifferent between taking the new drug and staying in their current health state. RESULTS: A total of 127 patients and 150 physicians completed the TTO questions. Among clinically-relevant levels of the health states for patients, cardiopulmonary arrest had the lowest utility (0.68), with serious hemorrhage (0.74), GI perforation (0.79) and rash (0.91) having higher utility values for utility. Utilities for physicians showed a similar pattern: cardiopulmonary arrest (0.75), serious hemorrhage (0.76), GI perforation (0.82) and rash (0.92). CONCLUSIONS: Results add to previously published literature regarding utilities for adverse outcomes from patients’ and physicians’ perspectives. Results show that patient and physician ratings of health states were largely consistent, suggesting agreement in the perceived impact of these adverse events.

PATIENT PREFERENCES FOR FIRST-LINE MAINTENANCE TREATMENTS FOR OVARIAN CANCER

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OBJECTIVES: Elicit medicine preferences of women eligible to receive first-line maintenance treatment for ovarian cancer and estimate benefit-risk trade-offs. METHODS: In the United States with self-reported physician diagnosis of ovarian cancer and eligible for maintenance therapy completed an online discrete-choice experiment (DCE) survey. The survey presented nine choice questions including five medical outcomes, four levels of quality of life, four levels of tolerability, and risks of side effects. Each profile was defined by the following attributes identified from the literature with clinical input and tested in patient interviews: progression-free survival (PFS), fatigue, diarrhoea, nausea and vomiting, hypertension, and risk of gastrointestinal (GI) perforation. The profiles in the choice questions were based on an experimental design with known statistical properties. Random-parameters logit was used to estimate preferences. RESULTS: Two hundred women completed the survey; median age was 49 years, 24% were late stage (3/4), and 44% had been diagnosed within 2 years. Across the attributes, better outcomes were significantly preferred to worse outcomes, except that responders did not distinguish between no nausea and mild nausea. Relative to the other attributes and levels, responders placed the greatest weight on avoiding severe diarrhea, followed by reducing the risk of GI perforation, and increased PFS. Respondents were willing to give up 6.5 months of PFS to reduce diarrhoea from severe to none. No statistical differences were found between the overall preferences of early versus late stage respondents, respondents above and below the median age in the sample, and respondents who had been diagnosed in the last 2 years compared to those diagnosed more than 2 years ago. CONCLUSIONS: Women with ovarian cancer were willing to trade-off efficacy (PFS) for improvements in side effects and risk. The lack of differences across subgroups suggest consistent preferences across the attributes within our sample. Funded by GSK.

PATIENT VERSUS GENERAL POPULATION PREFERENCES IN ANTICOAGUANT THERAPY

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OBJECTIVES: Evaluate preferences of patients to compare with preferences of the general population for anticoagulant therapy. METHODS: A DCE questionnaire was designed and administered eliciting preferences for benefits and risks. Seven attributes described hypothetical treatments randomly provided an "added benefit" to patients. Future clinical development plans should include well-designed comparative studies to improve likelihood of reimbursement and patient access.