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(PM1) CART ANALYSIS AS A TOOL TO DETERMINE OPTIMAL TREATMENT INTENSIFICATION TIME IN DIABETES

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OBJECTIVES: A key objective of treating type 2 diabetes mellitus (T2DM) is maintaining glycaemic control, (glycated haemoglobin-HbA1c) with targets set between 5.5% and 7% for patients treated intensively and between 7% and 8% for those treated with a less intense approach. This study aimed to assess the local clinical effects of this inertia. The study involved Classification and Regression Tree (CART) analyses to evaluate the time at which intensification should occur to gain glycaemic control.

METHODS: Incident T2DM patients were identified in the UK CPRD database between 1 January 2000-31 August 2014 and followed for 5 years. Patients initiated on metformin monotherapy were included. The optimal timepoint that intensification should occur was determined using CART. T2DM patients were reported in France and the UK were haematologists, versus 10% in Germany (where 80% were oncology-specialized) indicating a gap between typically recognized high-risk disease. The proportion of patients with high-risk disease based on ISS stage and cytogenetics was 18%-24% across countries. Bortezomib/thalidomide/lenalidomide (SCT)-eligible patients in France, but was unused in Germany and less common in the UK. The French advisors considered that the cost savings realized through informal care should be recognized in HTA if it can be related to patient benefit. Discussions in this area are increasing but changes to German advisors suggested that caregiver burden may be recognized in HTA if it can be related to patient benefit. Discussions in this area are increasing but changes to

research poster presentations – session V

PMS1

HOW TO CONDUCT ECONOMIC EVALUATIONS OF NEW TREATMENTS FOR ADVANCED CANCER WHEN OVERALL SURVIVAL DATA ARE NOT AVAILABLE?

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OBJECTIVES: Use of surrogate endpoints like progression-free survival (FFS) and time to progression (TTP) instead of overall survival (OS) in clinical trials for advanced cancer remains challenging against a health economic standpoint. This study assessed the use of surrogate endpoints in economic evaluations of antinecic drugs and methodological approaches adopted when reliable OS data are unavaiable.

METHODS: A systematic literature review was conducted to identify economic evaluations of treatments for advanced cancer published between January 2003 and October 2013. Cost-effectiveness and cost-utility analyses expressed in terms of cost per life-year gained and cost per quality-adjusted life-year using a surrogate endpoint as an outcome measure were eligible. Characteristics of selected studies were extracted and comprised: population, treatment of interest, comparator, line-of-treatment, study perspective, and time horizon. Use of surrogate endpoints and methods adopted when OS data were lacking were analyzed. Results: In total, 7,219 studies were identified and 100 fulfilled the eligibility criteria. Most included studies assessed the cost-effectiveness of a biological therapy (65%) in the first-line setting (56%) and in the context of advanced non-small cell lung cancer (24%) or advanced breast cancer (22%). Surrogate endpoints mostly used were FFS and TTP, accounting for 92% of included studies. OS data were unavailable for analysis in nearly 25% of economic evaluations. In the absence of OS data, studies most commonly assessed the effect of death. Of 477 reviewed patient populations, 288 studies used indirect comparison based on various assumptions, use of a surrogate endpoint as a proxy for SO, consultation with clinical experts, and use of OS data associated with the drug.

Conclusions: Although several approaches are used, there is no consensus method to estimate the cost-effectiveness of new anticancer drugs in the absence of reliable OS data.

PMS2

SELECTION OF STATISTICAL APPROACH IN UNDERSTANDING THE ROLE OF CONTRAST MEDIA IN INPATIENT INTERVENTIONAL CARDIOVASCULAR PROCEDURES

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OBJECTIVES: Bleeding episodes pose significant economic and quality-of-life (QoL) burdens on persons with hemophilia. This study aims to identify socio-demographic and clinical characteristics associated with annual bleeding frequency (ABF).

METHODS: Between 2005-2007 and 2009-2012, the Hemophilia Utilization Group Studies Va and Vb, respectively, recruited hemophila A or B patients from ten geographically diverse Hemophilia Treatment Centers in the United States. Adult patients or parents of children completed a baseline survey that collected information regarding socio-demographics, clinical characteristics, and treatment patterns. During the two-year observation period, bleeding episodes were collected through patient-reported follow-ups. ABF was determined from patient-reported bleeding episodes. Two-year drug dispensing information was also recorded and analyzed. Multivariable linear regressions were used to assess the association between baseline variables with ABF. Results were similar for all treatment groups. Other methods included use of indirect comparison based on various assumptions, use of a surrogate endpoint as a proxy for OS, consultation with clinical experts, and use of OS data associated with the drug.

Conclusions: Although several approaches are used, there is no consensus method to estimate the cost-effectiveness of new anticancer drugs in the absence of reliable OS data.
specification when the decision-making factors for selection of CM may vary from study illustrated the importance of controlling for observable and for unobservable characteristics. Late-Onset Alzheimer's disease patients, PS1, PCR – RFLP opportunities for AD therapeutic strategies.

49 AD patients and the subjects were categorized in two groups (14 Early-Onset AD and 35 Late- Onset AD) patients, called attention to the importance of genetic types among the Early-Onset AD and Late- Onset AD subjects demonstrated a significant difference in the mean number of accepted ME as proportion of all submitted additions; additional benefit based on accepted ME; percent of assessments in which at least one ME was accepted. RESULTS: In total, 19 oncology and 27 non-oncology assessments have been included into the analysis. For the 19 oncology assessments, a total of 76 ME had been submitted. IQWiG accepted hereof 20 ME (~26%). Accepted ME include: pain, skeletal-related complications and symptoms. The IQWiG determined an additional benefit for six assessments based on ME (6/19-32%). For the 27 non-oncology assessments, 127 ME had been submitted. IQWiG accepted 79 (~62%) including: strokes, cardiovascular events and relapse-related events. For non-oncology substances, IQWiG determined an additional benefit for thirteen assessments based on ME (13/27≈48%). At least one oncology endpoint was accepted in 83% of oncology assessments and in 100% of benefit assessments in all other indications. CONCLUSIONS: Though the rate of accepting ME in oncology indications is numerically lower (32% vs. 48%), the difference does not reach statistical significance (p=0.36 Fisher’s exact test).

PM69 COMPARISON OF TREATMENT-RELATED ADVERSE EVENTS RECORDED IN ADMINISTRATIVE CLAIMS DATA WITH THOSE RECORDED IN ELECTRONIC MEDICAL RECORDS FOR MULTIPLE MYELOMA PATIENTS

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OBJECTIVES: The decision to utilize a particular product may depend on formal hospital guidelines, physician practice patterns, negotiated reimbursement schedules with insurance companies, and other local (geographic and/or hospital) characteristics. These elements are mostly unobservable for the purpose of statistical inference. A hospital fixed-effects model specification, in essence, controls for the unobservable hospital time-invariant characteristics. Focusing on the relationship between iso-osmolar contrast media (CM) and low-osmolar CM agents (LOCM) and renal failure events in patients undergoing infrapatellar interventional procedures, this study highlights the importance of controlling for such factors by comparing statistical specifications with and without hospital fixed-effects. METHODS: The Premier database was used to identify patients between January 2008 and September 2013. Eligible patients had a primary procedure of angiography, which was defined as a fluoroscopy-based test and for which this database recorded fixed-effects. RESULTS: A total of 385,290 interventional procedure visits met the inclusion criteria. The unconditional univariate analysis showed 1.7% (p<0.01) more renal failure events in the iso-osmolar CM cohort compared to LOCM. The most saturated (OLS) cross-sectional analysis showed 1.4% (p<0.01) fewer renal events in the iso-osmolar CM cohort compared to patients who received LOCM. Controlling for unobserved heterogeneity across hospitals using the most saturated hospital fixed-effect specification, we found 2.3% (p<0.01) fewer renal failure events with iso-osmolar CM. CONCLUSIONS: This study illustrated the importance of controlling for observable and unobservable characteristics associated with these hospital characteristics. These elements are mostly unobservable for the purpose of statistical inference. A hospital fixed-effects model specification, in essence, controls for the unobservable hospital time-invariant characteristics. Focusing on the relationship between iso-osmolar contrast media (CM) and low-osmolar CM agents (LOCM) and renal failure events in patients undergoing infrapatellar interventional procedures, this study highlights the importance of controlling for such factors by comparing statistical specifications with and without hospital fixed-effects.