complement RCT data and prospective patient registries for the evaluation of contemporary practice including biomarkers used for diagnosis, treatment decisions and prognosis in the management of CML patients.

PCN148
THE USE OF PERSONALISED MEDICINE IN CANCER TRIALS

Wilson TJ, Hamerslag L, Kusel J, Brooks-Rooney C, Rolfe F, Costello S
Costello Medical Consulting Ltd., Cambridge, Cambridgeshire, UK

OBJECTIVES: The consideration of subgroup analyses is an emerging topic in health care evaluation. With value-for-money being an important issue, alongside the question "is this therapy effective?", another question becoming more relevant is "in whom is this therapy effective?" This issue is particularly relevant to the development of cancer treatments, which are often expensive and indicated in small patient populations. The use of personalised medicine is therefore expected to play a large role in this disease area. The aim of this study was to investigate how the proportion of cancer trials taking personalised medicine into account has changed over time. **METHODS:** ClinicalTrials.gov was searched for all interventional cancer trials that considered the use of individualised medicine, by using search terms including 'diagnostic', 'prognostic' and 'biomarker'. Search results were de-duplicated, and the start dates of these trials were analysed and compared to those of all interventional cancer trials listed on ClinicalTrials.gov. **RESULTS:** In total, 2810 cancer trials considered personalised medicine. The distribution of these was strongly skewed towards recent years, with only 57 of the trials identified having started before 2000. Across all cancer trials, 2.5% of those started before 2000 considered personalised medicine, whereas this percentage increased to 13.6% after this date. Interestingly, 20.6% of cancer trials commencing in 2010, compared to 17.0% of those in 2011, involved individualised medicine, indicating that there might be a slight decline in the investigation of personalised medicine recently. Trials considering individualised medicine were most often conducted in the United States or Europe, and in disease areas such as leukaemia, head and neck, brain, and prostate cancer. **CONCLUSIONS:** Personalised medicine has started to play a bigger role in cancer therapy development since the year 2000. With the current health care market focusing on value-for-money, however, it is surprising that only one-fifth of recent trials considered this issue.

PCN149
TRANSFERABILITY OF PHARMACOECONOMIC EVALUATIONS: CASE STUDY OF TRASTUZUMAB FOR EARLY BREAST CANCER

Carswell C, McWilliams P, Lyseng-Williamson KA, Faulds D
Adis, Auckland, New Zealand

OBJECTIVES: Using a simple method we determined the potential transferability of a previous economic evaluation on the cost effectiveness of adjuvant trastuzumab therapy for the treatment of HER2/Neu-positive breast cancer in Canada (Skedgel et al, 2009) to five other countries (UK, US, Australia, Japan and Germany). **METHODS:** Based on data from a literature review, we firstly identified all possible transferability factors. From this we selected key transferability factors – those with values that differed across the countries or were factors that were shown to influence the cost-effectiveness ratio in sensitivity analysis in the Canadian reference study. We then considered the ease of transferability (ranging from very low to very high) for each of these potential factors from the Canadian study to the other countries. **RESULTS:** We identified seven potential key factors for transferability: cost discount rate, health outcomes discount rate, unit costs (particularly drug acquisition cost), resources used, treatment effectiveness, (including duration of benefit) and measures used to determine utility values. Overall, potential transferability was highest for the UK, where treatment practice is similar to that in Canada and data on unit costs, resource use and discount rates are readily available. Because the authors of the reference study did not report unit costs and resource use separately, however, transferability of the analysis was hindered. Transferability to Australia, Germany and the United States was of an intermediate level, while transferability to the Japanese setting was the lowest because treatment practice is likely to be different, and little cost of illness and utility data exist for that country. **CONCLUSIONS:** Several key factors need to be considered when evaluating whether a study is transferable to another setting. To enable the transferability of economic evaluations from one country to another, authors need to ensure that they report their economic data clearly and in sufficient detail.

PCN150
DIRECT MEDICAL COSTS OF HEAD AND NECK CANCER IN THE UNITED STATES: AN ANALYSIS USING POOLED MEDICAL EXPENDITURE PANEL SURVEY (MEPS) DATA

Coughlan D, Frick KD
Johns Hopkins Bloomberg School of Public Health, Baltimore, MD, USA

OBJECTIVES: Pooling annual data together from the Medical Expenditure Panel Survey (MEPS) is legitimate way to produce average annual estimates based on "person-years" for any condition. AHRQ state that over 100 cases are required in order to do this. The objective of this study is to look at the direct medical costs associated with head and neck cancer (HNC) using this data source. **METHODS:** MEPS data was pooled (2003-2008) and analyzed for respondents with HNC (CCS code=11). Two different approaches were used. Consolidated year files and condition files were pooled together to calculate estimates on use and expenditures for persons with HNC (condition approach). Yearly event files were used to pool condition-event files to establish an attributable fraction approach. Both approaches inflated expenditure data to 2008 USD. **RESULTS:** A total of 120 respondents were identified to have a diagnosis of HNC when data was pooled. The condition approach estimated that the national yearly expenditures of HNC is in the order of $16.47bn with mean spend of $14,573 (SE±$2,227) per case per year. The attribut-

able fraction approach estimated that expenditures for all events associated with HNC are significantly less - $8.49bn with a mean of $4788 (SE±$1,057) per case per year. There were only 103 cases that had an event associated with the condition. Private payors accounted for most expenditure, though the proportion was slightly lower using the condition approach (46% vs. 56%). The analysis noted that attributable expenditures were driven by ambulatory visits where condition expenditures were driven by inpatient costs. **CONCLUSIONS:** MEPS is often used to estimate the direct medical costs of a condition. This analysis illustrates that for rare cases, such as HNC, that both approaches offer insight into characterizing a condition. Subsequently, a range for cost estimates can be determined using this data source.

PCN151
DESIGNING CASE REPORT FORMS FOR ECONOMIC EVALUATION ALONG SIDE CLINICAL TRIALS: A CASE-STUDY USING AN INTERACTIVE DATA ANALYSIS TOOL TO STREAMLINE DATA COLLECTION

Foley K
Thomson Reuters, Cambridge, MA, USA

OBJECTIVES: Determining which economic and health resource utilization data points to collect in clinical trials requires a balance between comprehensiveness and data collection burden. Cost and time constraints necessitate that only the most critical economic variables be collected. Our objective was to test the utility of a new tool for determining the most frequent types and timing of healthcare utilization among cancer patients in a quick and low cost manner. **METHODS:** We used an online interactive data analysis tool, *MarketScan*®*Treatment* Pathways, to explore the most frequent adverse events (AE) and their related healthcare utilization patterns in a sample of non-small cell cancer patients (NSCLC). Patients with at least 2 ICD-9 codes for lung cancer on different days within 30 days of each other on non-rule out claims and no chemotherapies associated with small-cell lung cancer were included. The subset of patients with a diagnosis for metastatic cancer following their NSCLC diagnosis who received at least one oral or injectible chemotherapy treatment were analyzed. **RESULTS:** 5,243 patients with metastatic NSCLC were identified, of whom 2,006 received at least one oral or injectible treatment. 80% of experienced at least one AE serious enough to require healthcare intervention. The median and mean days to the first AE were 20 and 51.5 days from the time of the first treatment. The most common AEs were anemia (51.2%), gastrointestinal events (34.8%), fatigue (26.1%), and neutropenia (24.2%). Of those with anemia, 36% received epoetin or darbepoetin alpha and of those with neutropenia, 77% received pegfilgrastim or filgrastim. Additional patient clinical and treatment characteristics were described for the 30 days following each AE. Total analysis time for this project was under 3 hours. **CONCLUSIONS:** Treatment Pathways answered critical questions for the design of economic endpoint data collection for a new cancer trial in just a few hours.

PCN153
A TRIAL FOR EVALUATING BREAST CANCER TUMOR MARKER USE IMPACT: A VALUE OF RESEARCH ANALYSIS

Thariani R[1], Blough DK[1], Barlow W[2], Henry NL[3], Gralow J[4], Ramsey S[5], Veenstra DL[6]
[1]University of Washington, Seattle, WA, USA, [2]Fred Hutchinson Cancer Research Center, Seattle, WA, USA, [3]University of Michigan Medical School, Ann Arbor, MI, USA, [4]Seattle Cancer Care Alliance, Seattle, WA, USA, [5]Fred Hutchinson Cancer Research Center, University of Washington, Seattle, WA, USA, [6]University of Washington, Pharmaceutical Outcomes Research and Policy Program, Seattle, WA, USA

OBJECTIVES: To assess the societal value of a prospective randomized clinical trial (RCT) for breast tumor marker testing in routine follow-up of high-risk, stage II-III breast cancer survivors. **METHODS:** We used value of information techniques to assess the benefits of reducing uncertainty of using breast cancer tumor markers. We developed a decision-analytic model of biomarker testing in addition to standard surveillance at follow-up appointments every 3-6 months for five years. Expected value of sample information (EVSI) was assessed over a range of trial sizes and assumptions. **RESULTS:** The overall value of research for an RCT involving 9000 women was $166 million (EVSI). The value of improved information characterizing the survival impact of tumor markers was $81 million, quality-of-life $38 million, and test performance $95 million. **CONCLUSIONS:** Despite not being recommended by clinical guidelines, the tumor markers carcinoembryonic antigen (CEA), cancer antigen (CA)15-3, and CA 27.29 are used by some clinicians to screen for increased risk of breast cancer recurrence. Although additional research may be warranted to evaluate the benefits and risks of breast cancer tumor marker tests, clinical trials would likely need to involve thousands of women and would take many years to complete. Our analysis indicates that substantial societal value may be gained by conducting a clinical trial evaluating the use of breast cancer tumor markers. The most important aspects of the trial in our analysis were information gained on survival improvements as well as quality-of-life parameters associated with testing and test sensitivity and specificity. Our analysis indicates that smaller randomized trials, as well as adding quality of life instruments to existing trials, retrospective, and observational trials can also generate valuable and relevant information.

PCN154
CHALLENGES POSED BY PATIENT CROSSOVER FOR COST-EFFECTIVENESS ANALYSIS OF ONCOLOGY PRODUCTS: A CASE STUDY IN METASTATIC PANCREATIC CANCER

Grima DT, Brown ST, Attard CL, Duong MT
Cornerstone Research Group, Burlington, ON, Canada

OBJECTIVES: Clinical trials of oncology products often allow crossover of control patients to the treatment arm following disease progression. This can underestimate a product's overall survival (OS) and raises challenges for cost-effectiveness analyses compared. **METHODS:** A lifetime model compared the cost-effectiveness of a hypothetical pancreatic cancer therapy (CRG001) to gemcitabine. Gemcitabine survival data were derived from published studies. A hazard ratio of 0.55 was assumed for CRG001. CRG001 cost $2500 every 2 weeks for a maximum of 12 cycles and gemcitabine cost $200 every 1 week for a maximum of 24 cycles. Analyses were conducted: 1) 0%; 2) 50%; and 3) 85% crossover of gemcitabine patients to CRG001. Patient crossover occurred at the time of disease progression. Crossover patients received the CRG001 hazard ratio. Patients progressing in CRG001 were assumed to receive palliative care. A secondary analysis allowed 50% crossover but excluded second-line costs of CRG001. Costs and outcomes were discounted at 5%. **RESULTS:** The cost per QALY gained for CRG001 compared with current care was $81,352 with no cross-over, $69,292 with 50% crossover and 40,992 with 85% crossover. In the analysis where the cost of CRG001 was excluded in crossover patients, the ratio was $140,118. **CONCLUSIONS:** The first three analyses illustrate that CRG001 cost-effectiveness decreases with increasing cross-over of gemcitabine patients, if the costs of CRG001 for crossover patients are included. In our experience, however, reimbursement agencies often require a primary analysis that excludes second-line costs of the study drug for patients that cross-over. This analysis yields a high ratio that could lead to a negative reimbursement decision. In this case, where second-line CRG001 costs are excluded, adjustment of OS for crossover of gemcitabine patients is required. Overall, consideration must be given to the extent and potential impact of crossover when conducting cost-effectiveness analysis of new oncology products.

PCN155

PATIENT BENEFIT-RISK PREFERENCES FOR ADVANCED RENAL CELL CARCINOMA TREATMENTS: RESULTS FROM A CONJOINT ANALYSIS STUDY

Mohamed A[1], Yang JC[1], Hauber AB[1], Liu Z[2], Wong M[3], Rogerio J[2], Garay C[2]
[1]RTI Health Solutions, Research Triangle Park, NC, USA, [2]Novartis Pharmaceuticals Corporation, East Hanover, NJ, USA, [3]University of Southern California, Los Angeles,, CA, USA

OBJECTIVES: To quantify patients' benefit-risk preferences for benefits, toxicities, and serious adverse events of advanced RCC treatments. **METHODS:** Adult residents in the United States, with a self-reported diagnosis of RCC completed a web-enabled choice-format conjoint survey consisting of a series of 10 treatment-choice questions, and a pair of hypothetical RCC medication profiles. Each profile had different attributes, i.e., efficacy [PFS], tolerability [fatigue, stomach problems, mouth sores, hand-foot syndrome (HFS)], serious adverse events (lung damage and liver failure), and mode of administration. Treatment-choice questions were based on a predetermined experimental design with known statistical properties. Random-parameters logit was used to estimate relative preference weights for each attribute level, mean relative importance weights; and calculate risk tolerance for each adverse event for different improvements in PFS. **RESULTS:** A total of 272 respondents completed the survey. A 7-month improvement in PFS was the most important attribute. Remaining attributes were ranked in decreasing order of importance: eliminating severe fatigue (7.0; 95% CI: 4.6-9.4), eliminating severe stomach problems (7.0; 95% CI: 4.7-9.3), eliminating a 2% liver-failure risk (6.1; 95% CI: 4.0-8.2), eliminating severe mouth sores (5.7; 95% CI: 3.7-7.7), eliminating severe HFS (4.5; 95% CI: 2.7-6.4), eliminating a 2% lung-damage risk (4.1; 95% CI: 2.5-5.8), and switching from infusion once a week to 1 pill once a day (2.5; 95% CI: 1.4-3.6). To increase PFS by 1 month (baseline: 3 - 4 months), patients accepted a maximum level of lung damage risk of 1.0% (95% CI: 0.8% - 1.4%) and liver failure risk of 0.7% (95% CI: 0.4% - 1.0%). A 7-month improvement in PFS was 2 times as important as eliminating severe HFS and a 2.0% risk of lung damage ($P < 0.05$). **CONCLUSIONS:** PFS was the most important outcome for RCC patients while severe fatigue and severe stomach problems were rated as the most troublesome toxicities.

PCN156

DEVELOPMENT AND VALIDATION OF A PATIENT-REPORTED QUESTIONNAIRE TO ASSESS THE QUALITY OF LIFE OUTCOMES OF INDIAN BREAST CANCER PATIENTS

Deshpande P, Chittkathopottamal AN, Bommareddy LS, Mallasamy S
Manipal University, Manipal, Karnatak, India

OBJECTIVES: To develop and validate a patient-reported questionnaire to assess the quality of life (QOL) outcomes of Indian breast cancer (BC) patients. **METHODS:** A 27-item questionnaire was developed by literature review, patient interviews (n=6) and expert opinions (n=15). The 24-item questionnaire was finalized. Total 11 domains were considered. The questionnaire was translated to local languages and then it was administered to BC patients (n=30) irrespective staging of cancer and type of therapy. The patients were interviewed and the responses were obtained. Internal consistency, acceptability, content validity, test-retest reliability of the questionnaire was determined and assessment the scores was performed statistically. **RESULTS:** A 24-item questionnaire was developed as per literature review, patient interviews and expert opinions. Cronbach's alpha value for the questionnaire was 0.93. Patients understood the questionnaire and found the items to be relevant indicating content validity. The statistical assessment of the scores was not showing the association between scores with age or stage of BC as sample size was less. **CONCLUSIONS:** The questionnaire shows good internal consistency, acceptability, content validity and test-retest reliability. It can be used to determine the QOL of BC patients. To our knowledge there is no other questionnaire to determine the QOL outcomes of Indian BC patients. For better results the instrument is needed to be used in larger population.

PCN157

TRANSLATION AND LINGUISTIC VALIDATION OF THE FACT-TH18 FOR USE WITH CANCER PATIENTS WITH THROMBOCYTOPENIA WORLDWIDE

Arnold B, Dhar J, Parks-Vernizzi E, Debb S
FACITtrans, Elmhurst, IL, USA

OBJECTIVES: Translation of patient reported outcomes (PRO) measures is an essential component of the research methodology required when preparing for multinational clinical trials. One such measure is the Functional Assessment of Cancer Therapy-Thrombocytopenia 18 questionnaire (FACT-Th18), which evaluates the quality of life (QOL) of cancer patients with thrombocytopenia. **METHODS:** This study set out to linguistically validate the FACT-Th18 scale for use in China, Greece, Hong Kong, Japan, India, Israel, Korea, Taiwan and Thailand. The combined sample consisted of 160 patients (81 males/79 females) diagnosed with thrombocytopenia. Patient mean age was 46 years, and at the time of administration, 146 patients were receiving treatment. The sample consisted of patients who speak Arabic, Chinese-Traditional, Chinese-Simplified, Greek, Gujarati, Hebrew, Hindi, Japanese, Kannada, Korean, Malayalam, Marathi, Punjabi, Tamil, Telugu and Thai. The FACT-Th18 was translated based on the established FACIT methodology. Patients completed the respective translated questionnaire corresponding to their primary language and then participated in a cognitive interview to determine if there were any problems with the translations or item content. Quantitative analyses were performed on the combined sample and participant comments were analyzed qualitatively in order to confirm the validity of the translations. **RESULTS:** During the translation process terms such as "petechiae", "pinpoint bruising" and "platelet transfusions" proved difficult to translate. The FACT-Th18 translations proved relevant to patients from a wide range of countries and were well understood. Very few items required adjustment to translations as a result of pilot-testing. **CONCLUSIONS:** The FACT-Th18 demonstrated linguistic validity across all 16 languages. The translations are considered acceptable for PRO assessment in international research and clinical trials.

PCN158

PROJECTING STATE LEVEL ESTIMATES FOR RARE DISEASE USING CENSUS DATA AND HEALTHE CARE CLAIMS DATABASE

Joo S[1], Victor T[2], Gomez A[3]
[1]Bristol-Myers Squibb Company, Pennington, NJ, USA, [2]Kantar Health, Princeton, NJ, USA, [3]Bristol-Myers Squibb Company, Princeton, NJ, USA

OBJECTIVES: Estimating prevalence rates for rare medical conditions such as renal cell carcinoma (RCC) at state level by age and sex is difficult due to the paucity of available data resources. Available information may be fragmented because of a lack of national level surveillance. The use of commercial medical claim data alone is insufficient for estimation because the use of these data tends to result in biased estimates due to business practices of managed care organization. **METHODS:** Invision Data Mart and the US census data were used to address this problem. The study inclusion criteria for defining RCC patients was age of 18 years or older without prior history of HIV/AIDS, HVB, or HVC diagnoses and had at least 2 outpatient medical claim with an associated ICD9 code of 189.0. First, we estimated prevalence rates for the medical conditions by state, age, and sex using ICD9 codes from the commercial data (2002-2010). Then, reanalyzed using post-stratification weights derived from the 2010 Census data to reflect the state, age, and sex distribution of the US population. **RESULTS:** The sum of the adjusted state population weights yielded a total that was similar to the 2010 US census data, and adjusted values suggest that the overall 2010 US RCC prevalence is approximately 85k. Since there is no state level prevalence information for RCC by age and sex available, an indirect comparison was made by comparing the overall prevalence from Kantar Health (CancerMpact®). The overall prevalence estimates were similar; Kantar Health: 86,853 versus Study Estimate: 84,712. **CONCLUSIONS:** This method produced prevalence rates that take important health care related factors into account in the estimation process. We recommend the use of this combined approach for the estimation of prevalence rates of rare disease conditions and procedures.

INFECTION – Clinical Outcomes Studies

PIN1

OUTCOMES ASSOCIATED WITH SEVERE COMORBIDITIES IN HOSPITALIZED CASES OF HIV/AIDS

Villa L[1], Skrepnek G[2]
[1]University of Arizona, Tucson, AZ, USA, [2]University of Arizona, College of Pharmacy, Tucson, AZ, USA

OBJECTIVES: To assess economic and clinical characteristics of severe comorbidities during inpatient hospitalizations in persons with HIV/AIDS. **METHODS:** The Agency for Healthcare Research and Quality (AHRQ) Healthcare Cost and Utilization Project (H-CUP) Nationwide Inpatient Sample (NIS) was used in this retrospective database study spanning 2005-2009. Inpatient cases of HIV/AIDS among persons 18 years of age or older were used as inclusion criteria. Key clinical comorbidities analyzed included organ insufficiency/failure, cancers, heart failure, pulmonary circulation disorders, coagulopathies, fluid/electrolyte disorders, and wasting syndromes/weight loss. Outcomes of inpatient mortality and hospital charges were assessed via multivariate logistic and gamma regression, respectively, after controlling for comorbidities, patient demographics, hospital characteristics, payer, and lengths of stay. **RESULTS:** There were 1,227,718 overall inpatient cases of HIV/AIDS from 2005-2009 in the United States, averaging 44.8 (±10.7) years of age, 6.7 (±9.0) days for length of stay, and inpatient charges of $36,004 (±59,303). Mortality occurred in 41,609 cases, constituting 3.4% of all HIV/AIDS hospitalizations and averaging 47.0 (±11.1) years of age, 13.4 (±17.4) days for length of stay, and inpatient charges of $104,558 (±136,254). Fluid/electrolyte disorders