several sources of real world data available to researchers. **METHODS:** We compare and contrast the pros and cons of data available from administrative (payment) databases, electronic medical record (EMR) databases, and surveys. **RESULTS:** Administrative claims databases provide fully-integrated, all-encounter patient data on diagnoses, procedures, and payments. However, data quality varies depending upon whether particular fields are required for provider payment. Data on lab and test values are typically lacking. Prescriptions that are written, but not filled by the patient, are usually not captured. Medical record data overlap, to a certain extent, with administrative data. While information on payments for services may not be included, detailed information on test results and lab values are usually captured in the EMR. Data are included on written prescriptions, but the researcher will not know whether the prescription was filled by the patient. Depending upon the clinical system covered, only some encounters (e.g., ambulatory care in the outpatient setting) may be available. Both administrative and EMR data hold the potential to provide longitudinal patient information that is not subject to recall or social desirability biases that often affect survey data. However, information on satisfaction with care, quality of life, activities of daily living, and many other metrics, may only be captured with survey data. **CONCLUSIONS:** Several sources of rich, longitudinal patient data are available to provide real world evidence on drug effectiveness and cost. In some cases, data may be combined to overcome limitations of a single source. With care, data may be found that will produce generalizable findings for the population of interest.

PRM34

ROUTINE DATA IN HTA: RECORD LINKAGE IN AUSTRIAS GAP-DRG DATABASE

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OBJECTIVES: Gathering data tends to be an expensive and time consuming task. During the IFEDH research project different methods for using routine data on models in HTA were discussed, improved and developed. Connecting a rich dataset from Austrias inpatient sector lacking patient identifiers and (kind of) personalized but sparse records from the outpatient sector provided by different social security institutions is the objective of this project. A detailed description of the setup and usage of the results were presented at the SHIP Conference 2011 in St. Andrews (http://www.scot-ship.ac.uk/conference-2011) and the International Data Linkage Conference 2012 in Perth (http://www.datalinkage2012.com.au/). **METHODS:** Documentation of prior processing and information of the provided data were not fully available and also questionable data quality and the presence of possible duplicates result in technical and contextual challenges. After prepossessing, data quality assessment and other preparations, a deterministic record linkage approach was developed using a combination of the open and freely available statistical environment R and PostgreSQL database. Based on dynamically created SQL statements and extensive logging, the linkage process can be enhanced easily if new knowledge about the input data gets available. **RESULTS:** The resulting linked dataset provides high quality and immediately available information. Additionally the deterministic linkage process can be examined and understood by its users. Therefore linkage and data errors are identified easily and feedback can be used to enhance the overall result. These experiences also lay the foundation for more advanced linkage methods and further improvements. **CONCLUSIONS:** After the long and challenging way from the first data import to a functioning data collection, adequate information can now be used in different projects with low costs and users confidence.

PRM35

PREVALENCE OF RARE DISEASES – A SPECIAL CHALLENGE FOR BENEFIT ASSESSMENT AND HEALTH ECONOMICS

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OBJECTIVES: With introduction of AMNOG legislation, the pharmaceutical companies must submit a dossier when launching new drugs. The dossier must include an estimate of number of patients to be treated. This is particularly challenging in case of rare diseases, as shown with transthyretin-type familial amyloid polyneuropathy (TTR-FAP). **METHODS:** Several sources were used for a comprehensive gathering of information. Apart from systematic literature research, incidence data was searched by internet research and within patient registers. Since current therapy of choice is liver transplantation, the German Organ Transplantation Foundation was consulted regarding frequency of liver transplantation in connection with TTR-FAP. Assuming all patients are immediately placed on a waiting list after diagnosis, the number of annually performed surgeries was equaled with the incidence of the TTR-FAP. Results of this research were compared to billing data of a national statutory health insurance. **RESULTS:** Given its European prevalence of approx. 1.1/100,000, TTR-FAP is an ultra-orphan-disease. For endemic regions (Sweden, Portugal) the disease is well captured and documented. For Germany, no comparable published data is available. 5 national treatment centers were identified that document their patients in various registers, reflecting an incidence of about 7 surgeries per year. According to the German Organ Transplantation Foundation on average 6 new TTR-FAP patients per annum were added to the waiting list, confirming the estimate of the incidence derived from registers. Domino transplantation is usual in TTR-FAP with an average of 6.5 domino transplantations p.a., validating the estimate of an incidence of 6 – 7 patients per year. Billing data reflects an estimated prevalence between 0.6 and 1.4/100,000. **CONCLUSIONS:** Data on the prevalence of rare diseases frequently is not available or very unreliable. Different sources produce strongly varying results due to several reasons. This must be taken into account when assessing the number of patients in the value dossiers for new drugs.

PRM36

PATIENT-REPORTED OUTCOME AND QUALITY OF LIFE INSTRUMENTS DATABASE (PROQOLID): EVOLUTION OF CONTENT, STRUCTURE, AND FUNCTIONALITIES (2002-2012)

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OBJECTIVES: PROQOLID was developed in 2002 to provide all those involved in health care evaluation with a comprehensive and unique source of information on Patient-Reported Outcome (PRO) and Health-Related Quality of Life (HRQL) measures available through the Internet. The objective of this study is to review the evolution of content, structure, and functionalities of PROQOLID since 2002. **METHODS:** The archives of PROQOLID were searched to retrieve the database just before its launch and to compare its content and structure as it was in April 2012. **RESULTS:** The first database, then known as QOLID, was retrieved. It included 313 instruments (32 generic and 281 disease- or condition-specific). Instruments specific to oncology were the most frequent (54). The structure had three categories: contact, conditions of use, and a copy of the questionnaire. By comparison, the April 2012 database includes 714 instruments (128% increase, with 100 generic and 614 specific), with an increase of almost 40 instruments each year (up by 401 in 10 1/2 years). Instruments specific to nervous system diseases are the most frequent (141), reflecting the evolution in the field (e.g., 76.7% of the neurological products authorized by the EMA have been approved since 2002). The information displayed for each instrument has been improved with the addition of five categories: translations available, descriptive information, content validity documentation, measurement properties, references, and websites. In January 2005 the database was renamed PROQOLID to reflect the wider use of the term PRO. In April 2012, a new Google-type search engine was added to make browsing more user friendly. Soon all users will have more information about instruments distributed by MAPI Research Trust. **CONCLUSIONS:** In just over 10 years, the PROQOLID database has considerably evolved in content and structure, and offers a range of information and services adapted to the evolution of the field.

PRM37

INDIRECT IDENTIFICATION OF PAYER PERCEPTIONS THROUGH RETROSPECTIVE ANALYSIS OF INTERVIEW WRITE-UPS

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OBJECTIVES: As part of consulting projects IMSCG performs >5000 payer interviews p.a. Interview analysis is typically focused on insights directly related to research objectives. However, often respondents provide perceptions/ unprompted insights unrelated to research objectives, which have never been analysed before. The objective was to test whether information obtained from unprompted payer responses in interview write-ups can be identified and analysed, to develop insights of payer perceptions of pharma and their impact on payer decisions. **METHODS:** We selected 100 projects performed 2008-2011 including double blinded interviews with national/regional payers/advisors, recorded in English in the IMSCG project database. Interviews were screened for quotes reflecting perceptions of pharma unrelated to the research objective. Only unprompted expressions were extracted and buzz-words created that allowed compression into a single word/phrase. Buzz-words were valued and categorized into perception clusters (reputation, interaction, employees, portfolio and research) to identify the cause of perceptions. Quotes linking perceptions directly to decisions were mapped separately. Respondents' function and geography were interlinked with the information. **RESULTS:** We identified 543 interviews including 900 quotes with unprompted expressions of perceptions in 1900 buzz-words. Distribution of interviews was equal between national/regional payers, but 80% of quotes came from regional payers. Top5 EU accounted for majority of quotes. Company specific and industry perceptions were evenly split. Data allows a variety of perception analysis: national vs. regional payer, pharma vs. company, company perceptions of national vs. regional payers. Perception clusters identify the cause of perceptions. Analysis of the impact of perception on payer decisions is feasible, based on 5% of overall quotes. **CONCLUSIONS:** The data allows a structured analysis of unprompted expressions of payer perceptions in retrospective research. Database processing is ongoing to derive in the future statistically significant answers related to the impact of payer perceptions on decisions and what these perceptions are based upon.

PRM38

ESTIMATION OF A MARKOV CHAIN FOR CROHN'S DISEASE AND CLASSIFICATION OF PATIENTS INTO DISEASE PHENOTYPES, IN EIGHT COUNTRIES USING INDIVIDUAL LONGITUDINAL DATA AGGREGATED OVER TIME

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OBJECTIVES: Crohn's disease is a chronic relapsing-remitting inflammatory bowel disease with heterogeneous disease course, requiring life-long treatment. Phenotypes explaining disease heterogeneity is of interest in optimizing allocation of health care resources, e.g. to avoid expensive maintenance treatment to prolong remission in patients who seldom relapse. To develop economic models for evaluation of treatments, our objective was to estimate parameters of a Markov chain from data on disease activity and resource consumption and to improve model fit by allowing different phenotypes. **METHODS:** We had individual data on relapse and remission, surgery, use of medicines and other resources, aggregated over three month periods, from inflammatory bowel disease patients from 1991 and ten year onwards. Data from Crohn's disease patients were extracted. An exact maximum likelihood estimator using observations aggregated over time was used to estimate monthly transition probabilities. This estimator was adjusted to allow different disease phenotypes using an Expectation-Maximization method which identifies the phenotypes that best describe patient heterogeneity. The estimated parameters were used to derive the mean durations of a relapse and a period of remission to describe the phenotypes. **RESULTS:** At least two distinct phenotypes were found in each country, seldom-relapsing (<once/3 years) and often-relapsing (>once/3 years). The best fit was with four phenotypes in Denmark, three phenotypes in the Netherlands and in Italy, and two in Norway, Israel, Ireland, Spain, and Greece. In Denmark and Italy there was a single seldom-relapsing phenotype and more than one often-relapsing phenotype. In Netherlands there was two seldom-relapsing phenotypes. Denmark, The Netherlands, Israel, Ireland and Italy have roughly as many seldom-relapsing as often-relapsing patients. Norway, Spain and Greece have a majority of seldom-relapsing patients. **CONCLUSIONS:** Allowing for different phenotypes improves model fit. Health care resource allocation can be optimized using phenotypes. Using data aggregated over time appears to remain a challenge.

PRM39

EVALUATION OF PATIENT CENTERED OUTCOMES USING INDIVIDUAL DATA FROM A QUALITY REGISTRY AND PATIENT REPORTED ABILITIES AND RATINGS OF QUALITY IN HEALTH CARE, IN DIABETES PATIENTS IN SWEDEN

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OBJECTIVES: The Swedish National Diabetes Register (NDR) has since 1996 longitudinally recorded medical outcomes e.g. risk factors, comorbidities, and covers the majority (80%) of Swedish diabetes patients. In addition patient reported values were collected alongside the registry in a questionnaire. Our purpose was to evaluate a method for measuring patient reported abilities and ratings of quality in health care and evaluating them together with outcomes extracted from the NDR. **METHODS:** A questionnaire was developed to measure if diabetes care is percieved as patient focused and efficient, through questions on self management ability, worries, ability to carry out daily acitivites, and perception of service, access and involvement. The questionnaire was issued to 4,760 patients, 2,916 responded. Registry data on risk factors (HbA1c, blood pressure, cholesterol) were extracted for each patient and connected to the questionnaire. Item Response Theory (IRT) was used to estimate patient abilities and patient ratings of quality in health care (IRT scores) from the response patterns. For each patient, registry data and IRT scores were used to derive an overall Malmquist approach output quantity index, a health care related component and a patient ability component. The index is a measurement of how efficiently the patient leads his or her life with diabetes and its care, and provides a measure of the patient's state of health in relation to the patient's situation. **RESULTS:** We obtained IRT scale models with good fit, satisfactory validated in another population. The IRT scores provide basis for patient evaluation in a broader perspective than risk factors alone. The ability index component varies more than the health care component. **CONCLUSIONS:** The questionnaire provides estimates of abilities and ratings of quality. Our approach allows estimating patient benefit and health care production using combined registry and patient reported data, the procedure probably easier for patients than methods like time trade-off.

PRM40

EFFICACY OF LIRAGLUTIDE COMPARED TO EXENATIDE AND INSULIN GLARGINE IN PATIENTS WITH DIABETES TYPE 2: A META-ANALYSIS

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OBJECTIVES: liraglutide and exenatide are the two known approved GLP-1 analogue drug in the management of diabetes, a network meta-analysis was performed to get a more robust evidence on the efficacy of liraglutide compared to exenatide in achieving HbA1c < 7.0% in more diabetic patient. **METHODS:** Electronic database was browsed for available material on the proposed subject until May 2012, the inclusion criteria were phase 3 randomise controlled trials in diabetes type 2 patients. The software ADDIS 1.14 (Aggregate Data Drug Information System) was used to perform the network meta-analysis of liraglutide, exenatide and insulin glargin. **RESULTS:** Node-splitting analyses showed that were no relevant inconsistency in the evidence. A consistency model was used to draw conclusion about the relative effect of the three treatments. The relative risk (RR) of liraglutide compared to exenatide is 1.28 (0.57, 2.82), RR of liraglutide compared to insulin glargine is 1.72 (0.70, 4.37) and the RR of exenatide compared to insulin glargine is 1.35 (0.66, 2.76). A vague prior for the study specific baseline (α) and the treatment effect coefficients (β) are $\alpha \sim$ N (0, 3.563E-3) and $\beta \sim$ N (0, 3.563E-3) respectively. The rank probability of the three drugs ranked liraglutide first, exenatide second and insulin glargine as the last in rank of the best treatments. **CONCLUSIONS:** Liraglutide is still effective in maintaining the HbA1c < 7.0% in more diabetes patients compared to exenatide and insulin glargine however exenatide once weekly seems to be more convenient to administer and has a cost advantage compared to liraglutide once daily dose. Liraglutide dose may need to be modified to once weekly or once monthly dose to me more effective in the management of diabetes type 2.

PRM41

DESIGNING PATIENT REGISTRIES: A CASE-STUDY USING AN ONLINE INTERACTIVE DATA ANALYSIS TOOL

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OBJECTIVES: Planning and designing patient registries requires significant research to determine the type and amount of data to collect, identifying recruitment sites, understanding the impact of study criteria on sample size, and estimating patient retention. Our objective was to test the utility of a new tool for answering these questions in a timely and cost-efficient manner, and to examine how claims data can be leveraged to plan registry design. **METHODS:** We used an online interactive data analysis tool, *MarketScan®Treatment Pathways*, to explore the characteristics and health care utilization patterns in a sample of cancer patients with pain. Patients newly diagnosed with prevalent cancers that are highly associated with pain such as multiple myeloma, colorectal, lung, prostate, or breast cancer were included, if they had at least 2 ICD-9 codes for one of the cancers on different days within 60 days of each other. A 6-month pre-period without any cancer diagnosis was used to identify new cancer patients. **RESULTS:** Of the 365,980 cancer patients meeting the entry criteria, 54% had an ICD-9 code for pain-related diagnosis. The median and mean number of days from cancer to pain diagnosis was 113 and 192 days, respectively. Only 3% had a co-morbidity that would exclude participation in the registry. Nearly 64% patients had an outpatient office visit within 30-days, of them, 68% had a subsequent visit in the following 30-days. Patient diagnoses, medications and procedures were described for the 60-day period following cancer pain diagnosis. The full analysis took 6 hours including all iterations on study criteria, and outputting descriptive data on patient demographic and clinical characteristics. **CONCLUSIONS:** Using *MarketScan®Treatment Pathways*, we tested sample selection criteria and health care utilization in a fraction of time than typical database analyses. These data answered critical questions in the study design for a planned cancer pain registry in a timely and cost-efficient way.

PRM42

MANAGING A SYSTEMATIC LITERATURE REVIEW PROJECT

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OBJECTIVES: A systematic literature review (SLR) is a well-established tool for identifying and assimilating existing evidence or identifying gaps that need to be filled by new research. Although SLRs are widely used in the drug reimbursement sphere, there are many challenges in maximizing its value and in communicating project objectives with a vendor. The objective of this study is to outline the deliverables of a SLR, and examine the optimal methodology in extracting maximum value from a SLR review by exploring important caveats and pitfalls of two hypothetical case studies. **METHODS:** Two hypothetical case studies are used to outline the process and the pitfalls of a SLR project and the relationship between industry and vendor. Feedback was elicited from consultants and industry in order to identify expectations and advice for a successful systematic literature review. **RESULTS:** The analysis found that in depth discussion during the protocol phase of the SLR is crucial to the success of the project. A successful protocol will incorporate: key questions that are focused and specific, scoping to outline the search strategy, and address the purpose of the review in terms of a product's value story (ie. a SLR for inclusion in a GVD), or evidence development. The analysis found that some challenges include too much or too little literature, which can be due to a very broad or narrow research question, challenges that arise due to expectations for certain data, and addressing gaps in the literature. Several suggestions on overcoming these challenges and caveats of the methodology are explored through the hypothetical case studies. **CONCLUSIONS:** The authors found that communication and a focused question were the most helpful in yielding successful literature reviews. Furthermore, detailed discussion at the protocol stage helped to avoid pitfalls at later points in SLR development. The authors provide a list of pitfalls and remedies that may help when considering SLRs.

RESEARCH ON METHODS - Modeling Methods

PRM43

A SYSTEMATIC REVIEW ON THE APPLICATION OF CARDIOVASCULAR RISK PREDICTION MODELS IN PHARMACOECONOMICS, WITH A FOCUS ON PRIMARY PREVENTION

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OBJECTIVES: In the absence of long-term randomized clinical trials (RCTs) on the effectiveness of pharmacological treatment for primary cardiovascular disease (CVD) prevention, risk prediction models are used to project changes in CVD incidence due to changes on risk factor levels observed in short-term RCTs. This study aims to summarize the literature on the application of these CVD risk models in pharmacoeconomic studies for primary CVD prevention interventions in high in-