View metadata, citation and similar papers at core.ac.uk
brought to you by CORE
provided by Elsevier - Publisher Connector



the user's behavior upon navigating the site. **RESULTS:** Since the site began to get monitored on July 1, 2012 and up until June 25, 2013, 19.738 hits were registered, with a monthly average of 1.661 hits. More than 65 countries accessed the site, the majority coming from the USA, UK and Portugal. Brazil leads with 93%. The rate of return of people who frequently accessed the site was 24,7%. Upon navigating the site, more than 45.000 visualizations of the web page were registered. **CONCLUSIONS:** This monitoring began in 2012 and therefore no previous years exist to serve as a basis for comparison. The results show that a considerable number of users have access to the site and consequently know about REBRATS. From the perspective of the website, the number of hits is still low, however, considering that the HTA field is a recent one in Brazil, its growth has been gradual. On an international level, new dissemination strategies are necessary in order to create greater visibility and promotion of the network.

**PRM47**

**DESCRIPTION OF TREATMENT PATHWAYS IN CHRONIC DISEASES USING LARGE GENERAL PRACTITIONER LONGITUDINAL DATA: THE EXAMPLE OF PHARMACOTHERAPY IN PARKINSON'S DISEASE IN THE UNITED KINGDOM**

Guelfucci F[1], Neine M[2], Bineau S[3], Milea D[3], Aballea S[2]
[1]*EPHE Sorbonne, Paris, France,* [2]*Creativ-Ceutical, Paris, France,* [3]*Lundbeck S.A.S., Issy-Les-Moulineaux, France*

**OBJECTIVES:** Identifying treatment pathways based on administrative data or electronic medical records is generally a complex task, as many incidental events may hide the key trends. The purpose of this study was to propose a transparent methodology for defining treatment pathways using a large longitudinal real life database. The application of two alternative algorithms with different variants in Parkinson's disease (PD) was described. **METHODS:** In the first method, a new treatment line was assumed to start when a pre-specified number of consecutive prescriptions of drugs from the same PD drug class or combination of classes (rasagiline, selegiline, dopa agonists, COMT inhibitors, levodopa and derivatives) occurred. In the second method, start and end dates of continuous treatment periods (i.e. without gaps higher than six months between prescriptions) were determined for each drug class independently of each other. Combinations were assumed to be used when different treatment periods overlapped. Algorithms were tested using medical records of patients with Parkinson's disease (PD) extracted from the UK Clinical Practice Research Datalink (CPRD). The outputs of the different algorithms were described in details for ten patients, randomly selected. The sensitivity of the algorithms to changes in assumptions was tested. **RESULTS:** The first algorithm did not systematically capture the addition of a new drug to the current treatment as combined drugs were not necessarily prescribed or renewed during the same consultations. However, the second method well captured the treatment lines observed but sometimes created undue treatment lines from isolated prescriptions. This issue was overcome by deleting short lines lasting less than three months. **CONCLUSIONS:** The second algorithm developed in this study provided an accurate description of the treatment strategies developed by prescribers in Parkinson's disease in the UK using a limited number of assumptions and may be useful for other chronic diseases.

**PRM48**

**IDENTIFICATION AND QUALITATIVE ASSESSMENT OF REAL WORLD DATA SOURCES: EXAMPLE OF LYMPHOMA AND MULTIPLE MYELOMA IN EUROPE**

Noïbi SO[1], Bertwistle D[1], MacDougall F[1], Berger K[1], Mehta J[2], Trask PC[2]
[1]*IMS Health, London, UK,* [2]*Sanofi, Cambridge, MA, USA*

**OBJECTIVES:** To identify and assess real world data sources for observational research on lymphoma and multiple myeloma (myeloma) in Europe. **METHODS:** Structured (EMBASE and MedLine) and grey literature searches were conducted to identify relevant European data sources. Identified data sources were screened based on the following criteria: inclusion of patient-level data and patients with NHL, HL, or MM diagnoses, possession of data dictionaries, and ongoing data accrual. Where regional registries were proven to feed data into national registries, only the national data source was retained for evaluation. Data sources passing initial screening were subjected to further evaluation based on publicly reported information, questionnaires and / or interviews with data source owners, and informed by lymphoma and myeloma treatment pathway analysis. **RESULTS:** One hundred eighty-six data sources from 21 countries were identified; of which cancer registries (R) accounted for 65%. The remaining 35% non-registry (NR) sources included biobanks, clinical audits, data linkage initiatives, drug databases and electronic medical records. Screening removed 101 sources. Of the 85 sources retained after screening, roughly half (52%) were NR sources and a majority (74%) were from six countries: France, Germany, Spain, Sweden, The Netherlands, and The UK. NR data sources generally collected more data attributes than R data sources. These data attributes included symptoms (collected by 52% NR, and 21% R data sources), treatment regimens (81% NR, 40% R), and resource use (49% NR, 8% R). **CONCLUSIONS:** Compared with disease registries, non-registry data sources in Europe typically have more diverse data attributes and are therefore potentially better for lymphoma and myeloma research. This result may guide the selection of data sources for observational research. As scrutiny of real-world outcomes for reimbursement of oncology drugs increases, much work remains to be done to increase the visibility and utility of data sources for health care payers and providers.

**PRM49**

**COVERING THE PASS: DEVELOPMENT OF PROTOCOL TEMPLATE LANGUAGE (PTL) FOR DATA COLLECTION AND DATA MANAGEMENT (DM) IN POST-AUTHORIZATION SAFETY STUDIES (PASS)**

Oberthur Johnson L[1], Watts H[1], Krishnan S[1], Zarotsky V[2], Spannheimer A[3], Siebenaler J[1], Gulyas S[1], Clark S[1]
[1]*OptumInsight, Eden Prairie, MN, USA,* [2]*Optum, Eden Prairie, MN, USA,* [3]*OptumInsight (Deutschland) GMBH, Munchen, Germany*

**OBJECTIVES:** To address non-interventional study (NIS) protocol DM and data collection requirements in PASS observational study designs. These studies may include =>1 cohorts, retrospective or prospective data collection, disease or treatment-based inclusion criteria, various outcomes (safety, prescription pattern or off-label use, comparative effectiveness, quality of life or other patient-reported outcomes[PRO], health economics, or other objectives), site/investigator selection (in-house, commercial, professional organization, or government databases), and country-specific requirements. **METHODS:** Studies that used integrated and non-integrated DM systems were evaluated to identify criteria for use and associated protocol template language (PTL), including required, preferred, or optional text based on various study designs and objectives. **RESULTS:** Multiple DM systems were evaluated and algorithms created with use criteria. For integrated DM systems that combined data capture and trial management, simplified PTL was identified. DM PTL was adjusted to include paper or electronic data capture (EDC) methods, paper/electronic-based PROs, paper/electronic case report forms (CRFs), data (eg. subject/physician entered, site or aggregate, medical or laboratory records) and appropriate subject data protection. For non-integrated systems that required separate DM and trial management, additional PTL was identified for these complex DM systems. The templates denoted minimum required language and suggested extended language for more elaborate designs. To support the numerous NIS study design types, we included decision trees to account for various study goals, associated outcomes, and data collection requirement. Our data collection decision tree summarized the variety of potential study designs with frequently used study goals/outcomes and associated data collection techniques. **CONCLUSIONS:** Integrated DM systems provided the most flexibility and simplicity of protocol template language. All template language was created as adjustable to meet the typical study designs encountered in PASS studies. This additional information was to support the protocol development team in creating the final protocol in a timely manner and in reviewing client-provided templates.

**PRM50**

**BUILDING A REAL WORLD PRACTICE-BASED NETWORK DATA PLATFORM TO LINK RARE DISEASE PATIENTS: CASE STUDY OF MYELOFIBROSIS PATIENTS IN THE UNITED STATES**

Lee A[1], Iqbal SU[1], Hess G[2], Fonseca E[3], Redmond S[3], Wang H[4], Rose M[5], Neumann F[6], Cohen P[6]
[1]*Sanofi, Cambridge, MA, USA,* [2]*Leonard Davis Institute, University of Penn, Philadelphia, PA, USA,* [3]*IMS Health, Plymouth Meeting, PA, USA,* [4]*Sanofi, Bridgewater, NJ, USA,* [5]*Sanofi, Chilly-Mazarin , Cedex, France,* [6]*Sanofi Oncology, Cambridge, MA, USA*

**OBJECTIVES:** Conducting real world evidence (RWE) research on patients with rare diseases is particularly challenging when using only one type of data (e.g. insurance claims). Myelofibrosis (MF) is a rare, hematological cancer with global annual incidence rate of <1 - 2.4 cases per 100,000 patients. MF is characterized by the reduced ability of bone marrow to synthesize blood cells which can result in anemia, thrombocytopenia, and higher risk of infection. Absent adequate, recent data on MF patients, a practice-based network (PBN) data platform was developed to facilitate RWE research on MF patients. **METHODS:** MF patients observed in IMS Health databases between November 2010 to October 2012 were identified. Academic centers of excellence and large community oncology practices treating MF patients were then identified to link additional patients into the platform using a HIPAA-compliant patient de-identification algorithm. Patient demographics and attributes of dispensed prescriptions, private practitioner visits, and electronic medical record data including laboratory information were collected across patients from January 2000 to March 2013. Descriptive analyses of demographic and clinical characteristics were conducted to assess the generalizability of the sample versus literature. **RESULTS:** A total of 6362 U.S. MF patients were identified in the platform. Mean (SD) age was 67 (12.6) and 45% of patients were female. Mean (SD) Charlson Comorbidity Index was 4.36 (2.4). Among the subset of 529 patients with laboratory results, 63% were identified as anemic and 34% had platelet counts 50,000 - 100,000 / microL. All U.S. geographic regions were represented. **CONCLUSIONS:** Demographic and clinical results suggest that this large sample of MF patients is comparable to prior estimates of the broader MF population. This case study of U.S. MF patients suggests it is important to look beyond any one data source and to build PBN platforms with key clinical domains spanning multiple geographic regions when conducting RWE research on patients with rare diseases.

**PRM51**

**FROM CLINICAL TRIAL TO REAL-WORLD EVIDENCE: A SYSTEMATIC APPROACH TO IDENTIFYING DATA SOURCES FOR OBSERVATIONAL RESEARCH**

Parasuraman B[1], Tomic KS[2], Winchester CC[3], Westergaard M[1], Johansson S[4]
[1]*AstraZeneca R&D, Wilmington, DE, USA,* [2]*Oxford PharmaGenesis™ Inc., Newtown, PA, USA,* [3]*Oxford PharmaGenesis™ Ltd., Oxford, UK,* [4]*AstraZeneca R&D, Mölndal, Sweden*

**OBJECTIVES:** Observational studies are often planned on an ad hoc basis, with the risk that methods are inconsistent and that aims overlap rather than complement each other. Our objective was to develop a systematic approach for identifying observational data sources for an integrated global programme of real-world evidence gathering, to support an important new indication for an antiplatelet drug. **METHODS:** Systematic literature and Web searches, supplemented with email and telephone contact with data owners, were used to identify and characterize registries and health care databases suitable for use in observational studies of myocardial infarction and acute coronary syndromes. The ability to identify patients across data sources was also assessed. Data were captured and evaluation criteria applied, including compatibility with aspects of an ongoing randomized clinical trial (patient population, treatments, outcomes, and length of follow-up [≥3 years]). Selection criteria included accessibility; availability of inpatient, outpatient, cardiac event, and drug data; and generalizability. **RESULTS:** Over 2700 publications were screened; we identified 216 registries and 380 databases (primarily of administrative claims and electronic medical records). Of these, 12 registries, and 21 databases met the evaluation criteria and were assessed in depth. After application of the selection criteria, 5 registries and 12 databases were recommended. Recommended data sources ranged in size, each capturing data on between 4000 and 11 million

patients, and were geographically diverse, representing populations in Europe, the USA, and Australia. Each recommended data source had unique strengths and limitations for use in real-world evidence studies, and together they had the potential to provide consistent and complementary information. The study output provided a valuable tool for global and local investigators. **CONCLUSIONS:** Observational data sources are diverse. A systematic understanding of real-world evidence can guide the development of a coherent strategy for designing observational studies to support clinical research.

**PRM52**

**FEASIBILITY OF USE OF MEDICAL TRANSCRIPTION DATA FOR REAL WORLD EVIDENCE GENERATION IN THE UNITED STATES: A PILOT STUDY OF PATIENTS WITH SYSTEMIC LUPUS ERYTHEMATOSUS INITIATED ON BELIMUMAB**

Narayanan S
*Ipsos Healthcare, Gaithersburg, MD, USA*

**OBJECTIVES:** To assess the feasibility of use of medical-transcription data for real-world clinical evidence generation in the US. **METHODS:** Majority of clinical practices in the US are experimenting digitization of patient medical records for two reasons: patient-management/care-delivery and billing/administrative/legal documentation purposes. Increasingly, physicians are using medical-transcription services, where physicians dictate details of patient visits and send the voice-recording-device to medical-transcription organization which processes voice-data and delivers electronic-files back to clinics; physicians review/revise/append the electronic-documentation and adds data to their patient-database-repository for future use. This pilot study assessed the utility of de-identified medical-transcription data in evaluating patient-physician dynamics and real-world treatment patterns/outcomes and documented adverse-events(AEs) using random set of adult SLE patients who initiated belimumab (a recently-launched biologic) within the past 2years as part of usual care. SLE was chosen specifically because of its complex clinical management issues. **RESULTS:** Nineteen belimumab patient transcription-records (de-identified) were reviewed (mean age:39.4yrs; female:95%). Top-4 clinical-manifestations at belimumab-start were: musculoskeletal(68%)/mucocutaneous(53%)/constitutional(42%)/renal(21%). Physicians discussed belimumab-attributes (specifically, infection-risks/AEs/etc), asked patients to do their own research on belimumab, and cited insurance/reimbursement-issues prior to belimumab-start in 58%, 26% & 26% of cases respectively; 11% of patients asked for belimumab. Top-3 documented-reasons for belimumab-start were: steroid-sparing(32%), control autoimmune diathesis(11%), control SLE-flares(16%). In 47% of patients, belimumab (at initiation) replaced another medication (majority:steroids/immunosuppressants); concomitant SLE-medications were: antimalarials(84%)/oral-steroids(68%)/Immunosuppressants(47%). Average belimumab-duration was 8.1months (overall data availability/patient:23.8months). During the observation period, 42% had >=1AE (e.g., diarrhea/rash/bronchiolitis/alopecia/upper-respiratory-track-infection), 42% discontinued belimumab (but 26% re-started) and 47% had some physician-documented improvement in outcomes (e.g., joint-pain/rash/energy level/fatigue). One patient had documented steroid-stoppage post-belimumab initiation. **CONCLUSIONS:** Medical-transcription data may provide documented real-world evidence of treatment dynamics and clinical status/outcomes associated with patient care. In this random cohort of SLE patients using belimumab, belimumab appears to provide some demonstrable benefits in almost half of the patients.

**PRM53**

**UPDATE OF THE PATIENT-REPORTED OUTCOME AND QUALITY OF LIFE INSTRUMENTS DATABASE (PROQOLID): INCLUSION OF E-PRO INFORMATION**

Caron M, Perrier LL, Acquadro C
*Mapi Research Trust, Lyon, France*

**OBJECTIVES:** In 2002, PROQOLID was launched to provide an overview of existing PRO instruments and a facilitated access to the instruments and their developers through the structured presentation of synthesized, reliable, and constantly updated data. With the constant growth of electronic capture of patient-reported outcomes, detailing information about modes of data collection (i.e., paper vs. electronic) in PROQOLID might become a crucial step in updating the database. The objective of this study was: (1) To review how e-PROs are currently reported in PROQOLID; and (2) To propose (if needed) ways of clarifying and updating e-PRO information. **METHODS:** PROQOLID was searched to retrieve current information about e-PROs using an advanced search engine. **RESULTS:** The e-PRO information was found under the category "mode of administration." Three options could be chosen: computer-administered, electronic-administered, and IVR (Interactive Voice Response) version. Out of the 751 instruments in the database, 37 (5%) were reported with e-PRO information. Information about existing translations of electronic versions was clearly specified for three questionnaires. To clarify and update e-PRO information in PROQOLID, several recommendations are proposed: (1) To create a new category, i.e., mode of data collection, in order to differentiate it from the "mode of administration" category; (2) To categorize each mode of data collection into five subcategories [i.e., Hand-Held Device, IVR, Internet web-data capture, Pen, and Tablet]; and (3) To provide a list of all translations available in each mode of data collection. **CONCLUSIONS:** This review has shown that PROQOLID already includes e-PRO information. Recommendations are given on how to modify the organization and content of the database to present the information on electronic capture of PROs.

**PRM54**

**USEFULNESS OF A COMMERCIAL HOSPITAL CLAIMS DATABASE TO IDENTIFY CURRENT ANTI-VASCULAR ENDOTHELIAL GROWTH FACTOR TREATMENT PATTERNS IN PATIENTS WITH EXUDATIVE AGE-RELATED MACULAR DEGENERATION IN JAPAN**

Narimatsu A, Adachi K, Wang ECY
*Bayer Yakuhin, Ltd., Tokyo, Japan*

**OBJECTIVES:** Access to large-scale public health claims databases is limited due to privacy concerns and lack of a unique identifier in Japan. Since such databases were not originally designed for outcomes research, we assessed the usefulness of one of the commercial databases in identifying treatment patterns in routine clinical practice. **METHODS:** Treatment patterns for patients with exudative age-related macular degeneration (AMD) using anti-vascular endothelial growth factor (anti-VEGF) was selected as an example to enhance our understanding of real-world clinical practice. A retrospective open-cohort study of patients diagnosed with AMD and treated from January 2010 to December 2012 at community hospitals employing Diagnostic Procedure Combination payment system was conducted by using hospital claims database provided by Medical Data Vision, Co., Ltd. Strengths and weaknesses of using the database were also identified. **RESULTS:** During the study period, 248 patients were diagnosed with AMD and received ranibizumab, 19 patients received pegaptanib, and two patients received aflibercept. Among the patients who received ranibizumab, the average number of ranibizumab injections per year was 3.21±2.38. The strength of the database was granularity of data including daily medication and medical procedural histories that allow us to obtain key parameters of analyzing treatment patterns. A major weakness was no unique identifier for patients; thus, a patient would be recorded as two different entries in the database if s/he visited two different hospitals. **CONCLUSIONS:** The results show that the commercial hospital claims database was useful to understand treatment patterns of exudative AMD at non-university hospital settings. Since treatment guidelines are usually written based on published clinical trial evidence, this type of database research provides an understanding of real life clinical practice and its associated patient outcomes, contributing to better adherence and future updates of treatment guidelines.

**PRM55**

**COMPARISON OF THE PRICES USED IN THE MANUFACTURERS' BUDGET IMPACT ANALYSES AND THE PRICES FROM REIMBURSED DRUGS LIST**

Iwanczuk T, Zawodnik S, Misiak J, Chodacka A, Sejbuk K
*Agency for Health Technology Assessment in Poland, Warsaw, Poland*

**OBJECTIVES:** To compare official prices and refunding limits of the selected drugs with the prices and limits used in the manufacturers' Budget Impact Analyses (BIAs) submitted in the reimbursement applications and in the manufacturers' documentations from request for removing, altering the level or method of financing of medicines to AHTAPol and to verify the accuracy of their assumptions. **METHODS:** Prices and limits proposed in BIAs in years 2007-2011 and official prices and limits for the same medicine were compared. Only BIAs for medicines which had been reimbursed for at least one year were included in the analysis. The official prices and limits were obtained from reimbursed drugs list published in Ministry of Health's Orders. **RESULTS:** In the studied period, 72 drugs were included in the analysis. Among them, 51,39% (37/72) were from reimbursement applications and 48,61% (35/72) were from requests for removing, altering the level or method of financing of medicines, respectively. As a result, 13% (9/72) of the prices proposed in the BIAs were underestimated. Median and mean difference (MD) between official prices and prices used in BIAs for the same medicine were 95% and 91% respectively. The overestimation was found in 39% BIAs (28/72); median = 117%, MD = 124%. Rest of the prices were equal 49% (35/72). Limits were underestimated in 8% (6/72) of the BIAs. Median and mean difference between official prices and prices used in BIAs were 97% and 98% respectively. Overestimation of the limits was found in 57% (41/72) of the BIAs; median = 133%, MD = 117%. Rest of the limits were equal 35% (25/72). **CONCLUSIONS:** Among the contradictory prices and limits official ones were higher than the prices assumed in BIAs for the same medicines. There is a strong need for further research.

**PRM56**

**DEVELOPING A PUBLICATION STRATEGY IN THE CONTEXT OF OUTCOME RESEARCH: A REVIEW OF THE LITERATURE**

Laloyaux C, Boyer M, Delbecque L
*Business & Decision Life Sciences, Brussels, Belgium*

**OBJECTIVES:** Many public health decisions are made based on information contained in medical publications. Therefore developing a publication strategy is a benefit for the pharmaceutical industry as it ensures an efficient dissemination of drug efficacy and safety evidence and an ethic development of the publications. In the context of outcome research, a publication strategy should take into consideration specific aspects related to the field (e.g., dealing with local, regional and global levels) and is important to ensure that unbiased information is provided to medical decision makers on time for the good of public health. A literature review was conducted to investigate if published guidelines on the development of a publication strategy in the context of outcome research exist. **METHODS:** A literature search of English articles was conducted on MEDLINE and Scopus. Search terms included "publication strategy" OR "publication planning" OR "publication coordination" AND "health economics" OR "health outcome" OR "outcome research" OR "medical economics". Independent extraction of articles was performed using predefined data fields. **RESULTS:** The search provided four citations; all were discarded after reviewing the abstracts. However, a simple search for the keywords "publication strategy" OR "publication planning" returned 85 hits in fields as various as psychology, political science, environmental science and medicine. None of the citations was specific to outcome research. **CONCLUSIONS:** The results of the literature search showed that the development of a publication strategy is an important concept through many different domains. However, no published guideline on the development of a publication strategy in the context of outcome research was found. A publication strategy specific to outcome research publications will be presented including recommendations regarding the key aspects that should be covered in an efficient outcome research publication strategy such as a critical assessment of published literature, identification of gaps, and development of a strategic plan.