opportunity cost. These issues informed the development of a new checklist, which was subsequently applied. Critical appraisals of cost-effectiveness studies should consider the aforementioned issues to conclude on their quality and potential to inform decision-making. More research is needed how to quantify the opportunity costs of complex interventions, particularly when multiple sectors are affected.

PRM257
THE VACCINE PORTFOLIO MANAGEMENT MODEL AS AN EFFICIENCY TOOL FOR JAPAN

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Health authorities may face a variety of options when deciding upon expanding their national pediatric immunization programs, ranging between an ad-hoc vaccine selection versus a targeted multi-year program to achieve efficiency goals. The vaccine portfolio management model allows the comparison of these two options over a fixed period of time. This optimization model is based on disease burden, vaccine impact and associated costs which are balanced against available vaccination budget and pre-defined public health priorities. Potential targets consist of reduction in disease events, or GP visits, or hospital occupancy rates, or deaths, or disease management cost. The model determines the optimal combination of vaccines selected per year, resulting in achieving the targeted public health outcome at the lowest annual budget. The financial results are then compared with those obtained after an ad-hoc selection of vaccines. The model was adapted for Japan in children up to 5 years old considering vaccines against pneumococcal disease, rotavirus, mumps and influenza disease based on published data. As an exemplary objective function we selected the reduction in hospital occupancy rates by 35% over a 5-year period. The portfolio model indicates that the optimal strategy consists of vaccination against rotavirus, influenza, and mumps at 90% coverage and 55% vaccine coverage against pneumococcal disease, requiring an annual budget of 331 million EUR. In case of a lower budget, the vaccine selection would prioritize first rotavirus, followed by influenza, then mumps and pneumococcal vaccine (depending on the available budget) to reduce hospital occupancy rates to a maximum extent. With an ad-hoc selection of vaccine introduction, the budget required to achieve the same objective function may increase by more than10% each year compared with the previous approach. A vaccine portfolio management model can therefore support decision makers in making efficient choices when expanding their national pediatric immunization programs.

PRM258
APPLYING SYMPTOM-BASED UTILITY FUNCTIONS IN HEALTH ECONOMIC MODELLING: A CASE STUDY OF UTERINE FIBROIDS

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BACKGROUND: A health economic model was developed to compare treatment strategies for uterine fibroids (UF). Bleeding and pain were identified as the principle disease-related symptoms affecting quality of life. Clinical trial data measured bleeding and pain through the pictorial bleeding assessment chart (PBAC) and visual analogue scale (VAS), respectively. However, the impact of PBAC and VAS on a single index measure of quality of life (QOL) is not widely studied. **OBJECTIVES:** Develop a symptom-driven utility function for patients with symptomatic UF, linking incremental changes in PBAC and VAS to a single index measure of QOL that enables the calculation of quality-adjusted life-years (QALYs). **METHODS:** PBAC, VAS, and EQ-5D levels based on clinical trial data were used in the analysis. Two ordinary least squares regressions (Reg1 and Reg2) were conducted with EQ-5D (UK value set) as the dependent variable. Reg1 (N=965) included PBAC and VAS as linear and quadratic terms to account for both linear and non-linear relationships. Reg2 (N=962) used a wider specification of independent variables including all regressors in Reg1 plus demographics and physical characteristics. **RESULTS:** The regression coefficients from Reg1 were -0.0001 (p<0.01) and -0.0044 (p<0.01) for a one-unit change in the linear components of PBAC and VAS respectively, an intercept of 0.9164, and adjusted R2 of 0.3135. The quadratic coefficient estimates were very small, positive, and not statistically significant. An increase in the linear components of PBAC (VAS) of 300 (30) results in a 0.0355 (0.1308) unit decrease in EQ-5D QALY-weight. Differences in the coefficients of PBAC and VAS on EQ-5D between Reg1 and Reg2 were small. **CONCLUSIONS:** The method described shows a pragmatic way to estimate QALY-weights in a health economic model that is responsive to incremental changes in patient symptoms for any intervention where PBAC and VAS data is available.

PRM259
QUALITATIVE DISCUSSION ON ISSUES OF PATIENT-REPORTED OUTCOME ASSESSMENT IN POST-MARKETING SURVEILLANCE FOR DISEASES IN THE ELDERLY

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Growing attention has been paid to patient-centric approach in clinical practice. The assessment of patient-reported outcomes (PROs) encourages communication between patients and physicians about goals of care. Post-marketing surveillance (PMS) is one of the available opportunities to collect large-scale, real-world data on patients' experiences and PROs. Since PROs must be answered by patients themselves by definition, elderly patients may face specific challenges and need extra support to conduct a PRO survey as part of PMS. Various difficulties in conducting a PRO survey in PMS are expected, such as: 1) Additional support required at the participating sites for elderly patients. For example, they may need someone to read out the questions or record the responses for them without influencing patients' responses 2) Lack of understanding of the value of PRO assessments for marketed

products among internal and/or external stakeholders, despite increasing use of PRO tools in regulatory studies 3) Maintenance of motivation and understanding of the study procedures in relevant healthcare professionals throughout the study period Key elements for best practices would include: 1) Strategize recruitment of physicians depending on their environment and resources available: general practitioners vs. hospital physicians 2) Deepen the understanding of the value of PRO assessments and contents of PRO questionnaires among internal/external stakeholders 3) Reinforce cross-functional collaboration in order to accelerate site recruitment, patient enrollment and the return of patient surveys We will further discuss the above difficulties during the planning and implementation phases of PMS with a PRO survey, as well as best practices based on our experience so far in Japan. These considerations and experiences will highlight important implications in conducting a PRO survey in PMS as a valuable opportunity to obtain real-world patient-relevant data in elderly patients immediately after a new product is available in the market.

PRM260
A COST-EFFECTIVE ENHANCED RETROSPECTIVE OBSERVATIONAL STUDY METHODOLOGY TO CAPTURE ECONOMIC BURDEN EVIDENCE IN A RARE DISEASE USING NON-TUBERCULOUS MYCOBACTERIA INFECTION AS A MODEL

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OBJECTIVE: Gaining adequate market access and reimbursement can be particularly challenging for orphan drugs. Barriers include inadequate real world data on the disease's epidemiology and its burden of illness. We deployed a unique combination of methodologies used in a multi-layered observational data capture approach to collect de-identified, publication-worthy clinical and resource utilization data in a probability sample of patients with a rare disease (pulmonary non-tuberculosis mycobacteria [PNTM]). We combine a new methodology ("blinded physician proportion survey") with a new use of an old methodology (Delphi expert survey). **METHODS:** First-round studies in France, Germany, Italy, Spain, and the United Kingdom consisted of a "blinded physician specialty proportion" survey to determine the probability of physician selection by specialty (2,585 participating physicians), a nationally representative chart review with participating physicians to determine target patients by region (619 physicians – 1,429 patients) and a Delphi study (an anonymous collaborative estimating methodology completed by six internationally recognized PNTM experts) to gain consensus on annual prevalence of PNTM for each target country. A second-round of survey and use of the collaborative estimating process is currently being completed consisting of a chart review with physicians of a nationally representative sample (n=30 per country) in a treatment refractory sub-group of PNTM patients to capture country specific treatment patterns and disease-related costs. "Refractory" is defined as at least one post-diagnostic positive culture despite 6 months of treatment. **RESULTS:** We developed a rigorous methodology to identify a sub-group population to address the gap of actual disease prevalence by country. Publication reviewers have consistently and congruently confirmed the first-round epidemiologic study methodology met their respective required scientific standards. **CONCLUSIONS:** Observational chart surveys in rare diseases that obtain a probability sample, a requirement for sample validity, can be used to provide essential disease-related metrics to populate market access and reimbursement evaluation procedures.

PRM261
A CONCEPTUAL SEARCH FILTER TO IDENTIFY REAL-WORLD EVIDENCE

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OBJECTIVES: Systematic reviewers utilise filters to focus searches of electronic databases to identify specific study designs. Established, tested search filters are available from groups which regularly conduct reviews such as the Cochrane Group, SIGN and CADATH. Although studies in the real-world are not new, novel phrases such as real-world evidence (RWE) are increasingly used to identify observational studies. Currently available search filters for observational and non-randomised studies do not adequately capture newer terms used. We have thus developed a method to identify frequently used MeSH terms in RWE studies and a search filter to include these terms. **METHODS:** A PubMed (MEDLINE®) search for RWE stated in the title/abstract was conducted. Articles with "real-world" and either "data", "evidence" or "research" in the title or abstract were selected; Case Reports, Comment, Editorial, Letter, News were removed. MeSH terms associated with articles were analysed and frequency counted; those relating to study design or outcome reporting were chosen for inclusion in the search filter. **RESULTS:** The MEDLINE® search identified 179 studies reporting RW and either data, evidence or research in the title. Of the 179, 151 were publication types of interest. The most frequently used MeSH terms related to RWE identified were 'Treatment Outcome' (n=30), 'Evidence-based Medicine' (n=17), 'Retrospective Studies' (n=15), 'Databases, Factual' (n=14), and 'Time Factors' (both n=14). A search strategy was developed combining MeSH and free-text terms to identify RWE. **CONCLUSIONS:** For every systematic review it is important to validate searches to ensure they are retrieving relevant studies; as new terminologies such as RWE are introduced to describe study design, reviewers need to adapt search filters. The method proposed allows searches to be adapted as terminologies are introduced and become more established.

PRM262
METHODOLOGICAL GUIDELINES FOR ECONOMIC DRUG EVALUATION STUDIES IN PORTUGAL: MAJOR GAPS AND NEW TOPICS IN THE STUDIES EVALUATED BETWEEN 2010 AND 2014

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