

demonstrated. Future research can use linked primary and secondary health care data to estimate the harm and cost associated with HPEs.

Multiple Diseases - Methodological & Statistical Research

PMU97 METHODOLOGICAL ADVANTAGES OF STANDARD ERROR OF MEASUREMENT FOR RESPONDER DEFINITION

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Objectives: Distribution-based statistics are often reported as supportive information to anchor-based estimates of responder definition (RD). The purpose of this study is to illustrate the difference among these statistics mathematically in terms of sensitivity and specificity to better facilitate the applications. **Methods:** Five distribution-based statistics (half standard deviation [SD], one standard error of measurement [SEM], 1.96 SEM, reliable change index [i.e., 2.772 SEM], and 4 SEM) were compared in terms of sensitivity and specificity rates under 24 conditions. Specifically, the conditions were generated based on 6 different reliabilities of instruments (i.e., $\rho=0.7, 0.75, 0.8, 0.85, 0.9, 0.95$) and 4 types of changes from baseline to post-treatment (i.e., no change, small change, moderate change, and large change). Based on the true-score model, the baseline and post-treatment scores were described as $Y_0=T+e_0$ and $Y_1=T+\delta+e_1$, respectively, where T denoted the true score, e denoted the error, and δ denoted a constant change. Assuming normal distribution of baseline and post-treatment scores, the sensitivity and specificity can be calculated using cumulative normal distribution function based on different conditions. **Results:** The conditions with no change provided the ground for calculating specificity of using different distribution-based statistics as RD. The specificities were 76%, 92%, 98%, and 99.8% for SEM-based statistics, and they were not affected by reliability. The specificities ranged from 74% to 95% for half SD, and the rates decreased with the increase of reliability. The conditions with true changes were used to calculate sensitivity. The sensitivity increased with the increase of change size and reliability in general. Based on the criterion of maximizing the sensitivity and specificity simultaneously, one SEM outperformed other distribution-based statistics. **Conclusions:** One SEM, derived from the scale characteristics, has methodological advantages when developing and interpreting RD.



PMU98 ELICITING UNCERTAINTY OF COMPLEX MODEL PARAMETERS: A CASE STUDY ON ELICITING THE TEMPORAL CHANGE IN THE TREATMENT EFFECT

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Objectives: Temporal uncertainty in cost-effectiveness modelling arises when the analysis time horizon exceeds the observed evidence end point. Quantifying the consequences of such uncertainty is often problematic. This study uses structured elicitation of experts' beliefs to characterise temporal uncertainty in the treatment effect, using a case study of a multifaceted podiatry intervention designed to reduce the rate of falls and fractures in the elderly. **Methods:** An elicitation exercise was designed and conducted to inform the temporal change in the effect of the podiatry intervention on the rate of falls and fractures. The elicited change in treatment effect was applied to the results of a randomised trial, REFORM, that reported at 1-year. The exercise was delivered using an online tool, designed to be completed remotely and independently to encourage experts' participation. **Results:** The study recruited 38 experts (geriatricians, general practitioners, physiotherapists, nurses, and academics) from across the UK. The elicited priors implied that uncertainty around the treatment effect, and thus the predicted outcomes, increased over time - the 95% confidence interval around the predicted rate of falls was 1.4-1.6 falls one year after starting treatment, compared to 0.7-1.6 falls after five years. The majority of experts believed that the treatment effect would depreciate over time, although beliefs varied across professions - geriatricians were least certain that the treatment effect would change, while nurses were the only experts who believed the treatment effect could potentiate. The differences could reflect the heterogeneity in their role in treating patients at risk of falling. **Conclusions:** The elicitation task was somewhat difficult for experts to complete and did rely on assumptions in order to make the exercise deliverable under the time and budget constraints. Nevertheless, the study demonstrates expert elicitation as a plausible method for informing uncertainty around the temporal change in the treatment effect, and illustrates the associated methodological challenges.



PMU99 A NEW APPROACH TO MEASURE EFFECTIVE COVERAGE FOR ASSESSING HEALTH SYSTEMS PERFORMANCE

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Background: Effective coverage (EC) is a measure of health service performance. The use of EC, however, is limited by the frequent lack of information about one of its constitutive parameters: the quality of health services, understood as the fraction



between observed and an optimum health gain from health interventions. We proposed a novel method that overcomes this issue. **Methods:** The procedure calculated the fraction of avoidable disability (or health-state utilities) in people receiving treatment, over the disability attributable to a disease. This was done using a regression model that predicts disability at individual level, where exposure to the disease without treatment, and with treatment (G2) were key variables. We compared total predicted disability in three scenarios: actual, worst ('0' coverage), and plausible (assuming quantile 90 of predicted health gain distribution) was the optimum performance in G2). We demonstrated this procedure using data from 4359 individuals aged 50+ at Wave 2 (2004-2005) of the English Longitudinal Study of Ageing (ELSA). Hypertension was used as an example disease. People reporting treatment were considered covered. Disability was measured as an index score, ranging from 0-100. **Results:** The prevalence of hypertension was 25.3% [23.9-26.5] and accounted for 1.9% [1.9-1.9] of total disability in the population. The coverage of treatment was 80.5% [78.2-82.9]. In people with hypertension, given current coverage, 48.8% [47.5-50.1] of the predicted disability in the worst scenario would have been avoided (i.e. relative-EC), and 2.0% [2.0-2.1] in the whole population (i.e. absolute-EC). The average health gain in people with treatment was 54.0% [52.9-55.0] (i.e. health benefit), while the avoidable disability against the optimum health gain was 68.6% [67.1-70.2] (i.e. quality). Effective coverage given the optimum health gain was 54.9% [53.6-56.1]. **Conclusion:** We developed a pragmatic way to estimate EC, which overcame the issue of the lack of information about health service quality.

PMU100 MEASURING IMPACTABILITY: A GAME CHANGER IN THE MANAGEMENT OF HIGH RISK MEMBERS - A BLUE CROSS BLUE SHIELD OF LOUISIANA PILOT

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Objectives: Health plans expend effort to try and predict the less than 1% of their members who will need extra support in order to maintain their health status by avoiding hospitalizations and emergency department visits. In addition to identifying high risk members, there must also be interventions available such as disease management programs and member participation in order to impact behavior and mitigate risk. This research pilots the development of a new metric termed "impactability" which is a score that evaluates a high risk members likelihood of benefiting from an intervention. **Methods:** Blue Cross impactability score was developed using machine learning algorithms with over 8,000 data elements such as patient demographics, credit score, family size, social support system, ZIP code, job title, location of workplace, and medical history. Data were combined with risk scores, social determinants, program evaluation results, and clinical feedback to create the impactability score housed in an enterprise wide data warehouse and made available to healthcare providers and Blue Cross case managers via a dashboard. **Results:** Preliminary results show that providers are increasing their engagement which is leading to more evidence based decision making. For example, a member that is over the age of 80 with end stage renal disease in hospice care is a high risk member; but his or her health status is not really impactable; whereas, a patient newly diagnosed with cancer is also a high risk patient, their impactability factor is high. There are care management programs that can be offered to mitigate this patients risk while reducing costs. **Conclusions:** The impactability score allows Blue Cross the ability to evaluate a patient's likelihood of benefiting from a particular intervention which in turn directs Blue Cross and providers to the right member, at the right time, through the best intervention approach maximizing care and reducing costs.



Multiple Diseases - Organizational Practices

PMU101 METHODS FOR ANALYZING THE TYPE AND TIMING OF LIFE SCIENCE INDUSTRY-FUNDED PUBLICATIONS

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Objectives: Explore whether product launch strategy can be elucidated by type and timing of industry-sponsored publications. **Methods:** In case #1, we identified a genomic diagnostic test company that publicly listed sponsored research on its tests. We categorized their publications as health economic and outcomes research (HEOR) studies, clinical trials, and *in vitro* studies. In case #2, we identified a pharmaceutical company that did not list research publicly. To identify sponsored research on a specific product, we developed a PubMed search strategy using company name as text word, limiting publication types to non-US government research support, and searching the condition of interest as MeSH terms or text words. We identified industry-sponsored research based on stated funding source and author affiliation. We used MEDLINE metadata to categorize publications by clinical trial phase and screened studies to identify HEOR. For both methods, we included full-length peer-reviewed English-language articles; we excluded non-human studies and opinions/commentaries. **Results:** In case #1 we screened 45 publicly listed studies sponsored on the diagnostic test, excluding 13 conference abstracts/commentaries. Of 32



eligible articles, 21 (66%) were HEOR, 6 (19%) clinical trials, and 5 (16%) *in vitro*. In case #2, our strategy identified 179 publications, 173 of which were industry-sponsored. Of 69 eligible studies, 65 (94%) were phase 1, 2, 3, or 4 clinical trials; 4 (6%) were HEOR studies. **Conclusions:** Publication strategy differed substantially between examples. Diagnostic methods other than the genomic-based test from case #1 exist, potentially leading the company to publish more HEOR than clinical and pre-clinical studies combined. The pharmaceutical product in case #2 had limited competition at launch, reducing the need to make an HEOR case. Our PubMed search strategy successfully identified industry-sponsored research, and we intend to use the strategy to develop a larger sample of cases.

PMU102 PERCEPTIONS OF THE APPROPRIATENESS OF CARE IN ADULT INTENSIVE CARE UNITS AT A BRAZILIAN TERTIARY ACADEMIC MEDICAL CENTER

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Objectives: Growing use of life-sustaining measures in patients with poor long-term expectation of survival concerns health practitioners with disproportionate care. Subsequent feelings of moral distress impact on quality of care of each new patient. Futile care also compromises allocation of scarce resources. We aimed to report prevalence of perceived inappropriate care among intensive care unit (ICU) staff physicians, training physicians, nurses and nursing auxiliaries in a public Brazilian hospital. **Methods:** Cross-sectional study of senior and junior doctors, nurses and nursing auxiliaries providing care to patients in the 34-bed multidisciplinary ICU of a tertiary teaching hospital in southern Brazil from January to July 2019. In total, 151 professionals completed an anonymous electronic survey. **Results:** Response rate was 49.5%. One-hundred (66.2%) respondents had more than 10-year experience on the field. One-hundred eighteen (78.1%) professionals believed there were disproportionate care at ICU. Both nurses and nursing auxiliaries were less likely than doctors to receive training on communication of end-of-life information (10.6% vs 57.6%, $p < 0.001$). Twenty-nine (28.1%) nurses and nursing auxiliaries versus 4 (0.08%) doctors claimed there was no palliative care deliberations in ICU ($p = 0.004$). Death was perceived as a treatment failure by 10 (0.06%) health care providers. Thirty-two (48.5%) nursing auxiliaries versus 8 (21.6%) nurses and 6 (12.5%) physicians affirmed all available treatment should be provided to patients regardless of their chance of survival ($p < 0.001$). Sixty-seven (44.3%) participants believed patients' wishes for palliative care were generally not respected. Forty-three (89.5%) senior and junior doctors believed collaboration between physicians and nurses was good, whereas 58 (56.3%) nurses and nursing auxiliaries disagreed on that ($p < 0.001$). **Conclusions:** This was the first survey regarding perception of inappropriate care conducted in Latin America. Although a unicentric study, it represents the views of healthcare providers who work at a high complexity medical center in a public health system.

PMU103 GLOBAL TRENDS IN ALTERNATIVE ACCESS ARRANGEMENTS FOR HIGH-COST ONCOLOGY AND RARE DISEASE DRUGS.

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Objectives: Last five years have seen the plethora of Alternative Access Arrangements (AAA) implemented globally by various payers, especially for high-cost Oncology and Rare Disease drugs. We evaluated the trends in such practices based on three different categories of US list prices. **Methods:** We used a non-systematic review of public data sources to identify various AAAs for Oncology and Rare Disease drugs categorized by the US list prices of 'less-than-\$100,000', 'between-\$100,000-and-\$250,000' and 'above-\$250,000'. Two broad categories – Financial-Based Agreements (FBA) and Outcomes-Based Agreements (OBA), constituted over 140 AAAs. **Results:** Two-thirds of the total agreements are FBAs, as discounts represent the quickest access solution to entry in most markets, but also skewed heavily by UK's focus on cost-effectiveness. However, concepts like 'free drug' and 'tiered pricing' are being tried sporadically in markets with limited experience with such approaches. The total number of OBAs is driven by the UK's reliance on post-market evidence development, particularly through the Cancer-Drug-Fund. Performance-based agreements are emerging in the US and Germany as opportunities to share risk while facilitating patient access. Italy continues to lead the way in OBAs due to a robust registry system, but details are not public. Truly innovative AAAs are more prevalent for drugs 'above-\$250,000', but payers still appear to be relying heavily on traditional approaches in such high-cost category. Other countries outside of US and EU are slowly experimenting with such novel AAAs as health system start maturing in many such countries (China, Brazil). **Conclusions:** Although biopharmaceutical manufacturers tend to price drugs based on the intrinsic value of the product, it appears that payer uncertainties around value of such products constitute a gap that necessitated the number and types of AAAs implemented. Robust discussion with

payers to manage such uncertainties via optimized clinical trial program can alleviate such gaps to facilitate patient access.

PMU104 UTILISING MOBILE TECHNOLOGY TO UNDERSTAND PHYSICIAN PRESCRIBING BEHAVIOURS CLOSE TO REAL TIME WITHIN PLAQUE PSORIASIS (PSO) AND ACTINIC KERATOSIS (AK)

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Objectives: Prescribing decisions can be difficult to understand in a 'real', uninterrupted way via traditional research methods. Direct questioning can lead to 'stock' answers; such as asking physicians to recall and explain prescribing behaviours where post-rationalisation can prevent an accurate read of drivers and barriers. **Methods:** To understand what drives plaque psoriasis and Actinic Keratosis (AK) physician prescribing and to identify the barriers between real and reported prescribing behaviours, LEO Pharma conducted a two-step, 8-week market research project involving close to 'real-time' decision capture methodology. Thirty-five primary and 16 secondary care UK physicians were surveyed using mobile App technology and telephone depth interviews (TDI) to ascertain what happens in the clinical context from first presentation of the patient through to prescribing of the treatment by the physician. **Results:** This report presents the findings of physician prescribing behaviour in patients with mild-moderate plaque psoriasis obtained from 30 TDIs and 213 primary care recorded videos. Four key drivers were identified from this research: most physicians have their own, unique default treatment pathway based on habit and familiarity, variation exists in how severity is defined and measured, patient distress influences how physicians measure severity and how aggressively they treat and finally, the patient voice is a powerful influencer with patient preferences and experience often determining the treatment prescribed. **Conclusions:** In conclusion, there is no standardised plaque psoriasis treatment pathway, but one which is individual to both the physician and the patient. Severity drives where new patients enter the pathway. Enabling the patient to express their level of distress could encourage physicians to treat more aggressively.

PMU105 A COMPARISON OF BIOPHARMACEUTICAL PRODUCT CHARACTERISTICS MOST SUITED FOR INNOVATIVE ACCESS AGREEMENT (IAA) IMPLEMENTATION IN THE US AND THE EU.

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Objectives: Emergence of high-cost biopharmaceutical products has increased the budgetary burden on payers, who responded with performance-based or financial schemes to provide optimal access. Across both single payer (EU) markets, and decentralized private payer markets (US), Innovative Access Arrangements (IAA) have been essential to manage uncertainties around value proposition of biopharmaceutical innovations. This research aimed to analyse the differences in implementation of IAA across US and EU. **Methods:** A non-systematic review of literature was used to construct a database of IAAs implemented between 2015 and 2019. Consultation with industry experts, review of payer communication was utilized to supplement the data. A list of product characteristics, such as first-in-class vs. follow-on entrant, value proposition over standard-of-care, potential follow-on indications over time horizon, as well as manufacturers' prior experience with innovative access agreements were evaluated and synthesized for each IAA. **Results:** We identified 67 IAAs (60% from US, 40% from EU) that were further evaluated based on above-mentioned product characteristics. Out of these, 18 (27%) were in Oncology, followed by 10 (15%) each in Virology, Cardiology & Diabetes while CNS and Rare Disease led the rest. IAAs in oncology were observed predominantly in EU, while the rest were implemented in the US. First-in-class products constituted over 75% of IAAs with a balanced split between the continents and tend to have more performance-based agreements (86%) to capitalize on its innovativeness. Additional analyses are ongoing to identify product characteristics such as extent of improvement over standard-of-care, robustness of efficacy data to postulate a set of characteristics for respective IAAs. **Conclusions:** Payers in the US and the EU follow different strategies and priorities in implementation of IAAs based on budgetary burden and uncertainty around product value. Engaging payers in line with their preferred IAA approach to address key objections can help create more customized arrangements to optimize patient access.

Multiple Diseases - Patient-Centered Research

PMU107 CONTEMPORARY TRENDS IN USE OF PATIENT-REPORTED OUTCOME MEASURES OF HEALTH IN CLINICAL TRIALS

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