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GENDER-SPECIFIC SAFETY OUTCOME REPORTS FOR NEW MOLECULAR ENTITY DRUGS
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OBJECTIVES: To describe reporting rates of gender-specific safety outcomes for new molecular entity (NME) drugs approved from the US Food and Drug Administration (US FDA) between 2009 and 2013 **METHODS:** New drug application (NDA) files for all NME drugs that were approved for systemic use in men and women were surveyed from the US FDA database. Specific reviews and key word searches were made for descriptions or tabulation of gender-specific inferential statistics on the drug safety section. The rates of inclusion of explicit descriptions or reports of statistical significance were calculated. **RESULTS:** Of all 457 approved drugs during the study period, 118 NMEs were included for the evaluation. Of those, 8 NMEs and 13 NMEs were excluded due to gender specific indications or non-systemic route of administration, respectively, thereby remaining 97 NME drugs were further reviewed. On average, gender-specific safety outcome descriptions were included in 74.2% (72/97) of the reviews. Gender-specific statistical significance was reported in 13% of the reviews. Format of the outcome descriptions was inconsistent varying from statement in the body text to table without descriptions. **CONCLUSIONS:** About one in four NDA reviews for NMEs was lacking sufficient descriptions on gender-specific safety outcomes and the format of the safety outcome reports by gender was inconsistent.

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IMPROVEMENTS IN SELF-REPORTED REAL-WORLD DECISION-MAKING ABILITY AFTER COMPLETION OF A COMPARATIVE EFFECTIVENESS RESEARCH CONTINUING EDUCATION CERTIFICATE
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OBJECTIVES: Little guidance exists for healthcare professionals on how to evaluate and use CER studies with new unfamiliar designs or methods. To fill this gap, the CER Collaborative (AMCP, IPSOR, and NPC) developed task force reports and on-line tools. The aim of this study was to determine if a multi-media continuing education (CE) program, largely derived from task-force reports and related online CER Collaborative tools, improved learner ability and confidence to assess CER studies for use in real-world decision-making. **METHODS:** Twenty healthcare professionals registered for a 19-hour, ACPE-approved CER Certificate Program (CCP) which included five self-paced, online modules and case studies: prospective and retrospective observational studies, indirect treatment comparisons, models, and synthesizing information from studies with various designs. A final live workshop was conducted to enhance learner skills through case presentation and peer critique. After CCP completion, learners assessed their ability to evaluate CER studies using a Likert scale (1= strongly disagree, 5=strongly agree). **RESULTS:** In the first cohort of learners, 18 (90%) completed the program on schedule. Significant improvement in self-reported ability to evaluate CER study design on their relevance and credibility ranged from 29.17% (CI 18.50 - 39.83) to 61.76% (CI 49.08 – 74.45) at program completion. Learners indicated high confidence in their CER evidence assessment abilities (mean score 4.17; CI 3.93 – 4.40). **CONCLUSIONS:** As new or unfamiliar CER study designs and analyses become available, there will be an increasing need for clinicians and other decision-makers to understanding how to assess the relevance and credibility of CER studies to inform decision-making. The CER Certificate Program, was associated with improved healthcare decision-makers' self-reported ability to evaluate CER and apply it for use in decision-making.

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PERCEPTIONS OF THE ROLES OF PATIENTS AND PAYERS IN PHARMACEUTICAL MARKET ACCESS
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OBJECTIVES: A review of the literature was conducted to determine whether there is an appropriate definition for market access. The original perception was that it relates to securing market authorization, as well as enabling prescribing of the product. A working definition for pharmaceutical market access was proposed as "a process of enabling patient access to pharmaceutical products, not only through gaining regulatory approval, but also through gaining an acceptable optimal price and reimbursement status to manufacturers and payers." The objective of this survey was to determine how appropriate the proposed definition is by comparing it to definitions proposed by differing professionals within the healthcare industry. **METHODS:** A review of the literature was undertaken, followed by the development of a questionnaire aimed at eliciting the various determinants of pharmaceutical market access, both from the patient and payer perspectives. This survey was administered to professionals in three settings involving ISPOR European Congress, EMAUD educational course, and a pharmaceutical company, with forty eight, forty five, and seventeen respondents, respectively. The respondents were categorized as belonging to: academia, pharmaceutical industry, healthcare profession, policy maker/Health Technology Assessment Agency (payer), and consultancy. Responses were recorded verbatim, and then coded by the interviewers to aid analysis. **RESULTS:** The survey suggested there is an inadequate understanding of market access, and this is independent of the professional background of the respondent. Furthermore, there is not a consistent agreement as to what factors influence the successful development and commercialization of pharmaceutical products. **CONCLUSIONS:** Successful market access of pharmaceutical products requires a wider understanding of the role of patients and payers in the development and commercialization by all stakeholders in the healthcare delivery system. Further research is needed into this field as it has important health policy implications for patient care.

HEALTH CARE USE & POLICY STUDIES – Health Technology Assessment Programs

PHP136
TREND ANALYSIS OF TECHNOLOGY APPRAISAL DECISIONS FROM THE NATIONAL INSTITUTE OF HEALTH AND CARE EXCELLENCE (NICE) – WHAT FACTORS INFLUENCE THE LIKELIHOOD OF RECOMMENDATIONS?
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OBJECTIVES: The objective of the analysis was to compare trends of single versus multiple technology appraisals (STA versus MTA) recommendations for new technologies conducted by NICE. Analysis was also conducted for products with European Union (EU) orphan designation. Further analysis was conducted to identify any disease areas that could be particularly challenging for companies planning a European launch of any new products, aiming to support planning for sequencing across the EU. **METHODS:** A longitudinal database containing all technology appraisal guidance published by NICE since the formation of the organization was analyzed. Analysis of products by disease area was conducted by classification into British National Formulary (BNF) categories. All published guidance was included thus reviews of technology appraisals, which overwrite earlier guidance, were not accounted for. **RESULTS:** In 2000-2007, the ratio of guidance published for MTAs to STAs was 3:1 (54 versus 17). However, this ratio was reversed in 2008-2014, when guidance was published for 46 MTAs and 132 STAs. In 2009, 92% of STA guidance included positive or restricted recommendation (n=12), decreasing to a historical low of 52% in 2013 (n=27) and increasing to 85% in 2014 (n=20). 50% of recommendations for orphan products were positive or restricted in 2011 and 2013 (n=6) but 100% were positive or restricted in 2012 and 2014 (n=3). The overall recommendation rate is lower for orphan products than no-orphan products (67% versus 73%). Malignant disease and immunosuppression treatments were the most common STAs but had the second lowest recommendation rate (59%, n=71). Cardiovascular treatments were most consistently recommended (100%, n=19). **CONCLUSIONS:** A decreasing proportion of appraisals include multiple technologies. There have been substantial variations in annual recommendations from STAs, possibly due to the choice of appraisal committee and evidence review group, which makes it very challenging for manufacturers to predict likely outcomes.

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HAVE CHANGES TO THE SCOTTISH MEDICINES CONSORTIUM (SMC) PROCESS FOLLOWING THE ROUTLEDGE REVIEW REALLY IMPROVED ACCESS TO ORPHAN MEDICATIONS AND WHAT FACTORS INFLUENCE RECOMMENDATIONS?
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OBJECTIVES: Data published by the Office for Health Economics in 2011 showed rejection from the SMC was more likely for orphan than for non-orphan products. Following a review of the SMC in 2012, the process for appraising end-of-life, orphan and ultra-orphan drugs was modified to facilitate greater access for patients in Scotland. We analyze if the revised SMC process has been successful in providing more positive recommendations for orphan products and which factors may influence the decision. **METHODS:** Analyses were based on a validated, longitudinal database of all recommendations from 2002 to 2014. Products with a European Orphan designation were analysed. SMC recommendations following full submission, resubmission or abbreviated submission were reviewed and sub divided into British National Formulary (BNF) category. **RESULTS:** In 2002-2014, there were 8 abbreviated submissions to the SMC for orphan products, 68 full submissions and 25 resubmissions. The positive recommendations rate (with or without restriction) from 2002-2013 was 53% (n=92); in 2014 this recommendation rate increased to 89% (n=9). Up to 2013, malignant disease and immunosuppressive treatments accounted for 43% of the published guidance of orphan products with a recommendation rate of 50% (n=40); in 2014 this improved to 80% (n=5). Cardiovascular treatments have a high recommendation rate of 92% (n=13) but treatments for musculoskeletal and joint diseases have the lowest success with all three submissions receiving negative recommendation. In 2013, evidence for 7 orphan products was not submitted to the SMC within the required 3 months of license thus received automatic negative recommendation, however, this reduced to only one non-submission in 2014. **CONCLUSIONS:** The SMC recommendation rate for orphan products, particularly malignant disease and immunosuppressive drugs, has improved from 2013 to 2014 suggesting the revised SMC appraisal process may be more effective in enabling the SMC to provide positive recommendations for orphan products.

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TREND ANALYSIS OF SMC DECISIONS – WHAT FACTORS INFLUENCE THE LIKELIHOOD OF POSITIVE, RESTRICTED AND NEGATIVE RECOMMENDATIONS?
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OBJECTIVES: Since formation in 2002, the Scottish Medicines Consortium (SMC) has evaluated 608 health technology assessment (HTA) submissions of new medicines for use in Scotland. They have, therefore, reviewed more clinical and cost-effectiveness evidence for medicines than any other HTA organization in the world. Trends in SMC guidance were analysed to provide insights on likely recommendations of new products as manufacturers navigate the UK market. **METHODS:** All analyses were based on a validated, longitudinal database of all published guidance from 2002 to 2014. SMC recommendations following full submission, resubmissions or abbreviated submissions were reviewed and then subdivided into British National Formulary (BNF) category. **RESULTS:** From 2002 to 2014, the SMC has published guidance following 608 full submissions, 232 abbreviated submissions, 157 non-submissions, 158 resubmissions and 5 Independent review panels. The proportion of products not recommended has fallen from 40% in 2007 (n=98) to only 17% in 2014 (n=84). Products falling into the malignant disease and immunosuppression BNF category were the most common submission to the SMC (20% of submissions, n=197) with an approval rate steadily