

OP29 Building A Global, Public Repository Of Patient Experience Data

Catherine Holliday (cmholliday@cc-dr.org)

Introduction. The Patient Experience, Expectations and Knowledge (PEEK) protocol was developed so that a holistic, comprehensive, independent, proactive and systematic approach could be taken to inform decisions made in the context of health technology assessment and other parts of the health sector. Each PEEK study is made publicly available which over time will result in a global repository of patient experience data.

Methods. The PEEK protocol is a single protocol that can be implemented across disease areas and includes a quantitative and qualitative component. The quantitative component is based on a series of validated tools that provide baseline health and demographic data for the study population. The qualitative component is the result of two years of protocol testing to develop a structured interview that solicits comprehensive and holistic patient experience data, and provides participants with the opportunity to provide advice on their future expectations.

Results. PEEK studies in breast cancer, bladder cancer, lung cancer, spinal muscular atrophy, atopic dermatitis, chronic kidney disease, chronic heart failure and mitochondrial disease have been completed in the Australian context (www.cc-dr.org/peek). Holistic patient experience themes are presented commencing with symptoms and diagnosis experience, through to communication, information, treatments experienced and quality of life. Information is also available in relation to participant's expectations of future treatment, care, information and communication. The result is a freely available repository of patient experience data that anyone in the sector can access to complement clinical and economic evidence.

Conclusions. The process of providing patient feedback and real-world evidence in the context of health technology assessment is often ad-hoc. The lack of consistency means that it has been difficult to assess the impact of patient engagement and feedback in the context of health technology assessment. The PEEK protocol and program is an example of a systematic, independent and holistic approach to patient experience and real-world evidence data collection that provides the sector with an opportunity to proactively engage the community in decisions that are made about treatment, care and support.

OP30 Impact Of Patient Reported Outcomes Data On Health Technology Assessments Of Acute Myeloid Leukemia

Manasee Shah (manasee.shah.contractor@astellas.com), Rianne Ernst, Stefan Holmstrom and Inge van Hooijdonk

Introduction. Patient-reported outcomes (PRO) data are important in understanding patients' experience of disease and treatment; however, PRO data are not universally collected or consistently included as part of a Health Technology

Assessment (HTA) submission. Additionally, the HTA bodies' response to PRO data vary, making the impact unclear. To understand the impact of PRO data on reimbursement decisions for Acute Myeloid Leukemia (AML) indications, an in-depth analysis of HTA bodies' appraisals of AML and analogous indications was conducted.

Methods. This analysis was conducted using IQVIA's HTA Accelerator, which contains HTA appraisals from ≥ 100 HTA agencies in thirty-nine countries. Included in the analysis were single-technology assessments (original submissions, resubmissions, extensions of original indications, and renewals); relevant regulatory approvals and pivotal trials were also analyzed.

Results. Of the 185 AML appraisals from sixteen HTA bodies, 66 (36%) included PRO data. Within these, thirteen different PRO instruments were identified, none of which have been validated in patients with AML. For seven of twenty in-scope products, PRO evidence positively impacted ≥ 1 of the HTA decisions. Although the same HTA bodies (i.e., Scottish Medicines Consortium, pan-Canadian Oncology Drug Review, and the National Institute of Health and Care Excellence) generally accepted the PRO evidence, others were critical of the evidence (i.e., Haute Autorité de Santé and the Institut für Qualität und Wirtschaftlichkeit im Gesundheitswesen). The most common concerns raised by the HTA bodies regarding the PRO evidence included trial design and low patient response rate.

Conclusions. Of the products that included PRO evidence in their HTA submissions, 35% received positive feedback from ≥ 1 HTA body on their submitted PRO evidence. Attention to PRO data collection is key to demonstrate the value of AML products to HTA bodies. Without these data, a clear gap in the understanding of patients' experience is evident.

OP31 HTA And Patients' Preferences: Starting A Discrete Choice Experiment

Thomas Poder (thomas.poder@usherbrooke.ca), Marion Beffarat, Maria Benkhalti, Pierre Dagenais and Ginette Ladouceur

Introduction. Hospital-based health technology assessment (HB-HTA) needs to consider all relevant data to help decision-making, including patients' preferences. In this study, we comprehensively describe the process of identification, refinement and selection of attributes and levels for a discrete choice experiment (DCE).

Methods. A mixed-methods design was used to identify attributes and levels explaining low back pain (LBP) patients' choice for a non-surgical treatment. This design combined a systematic literature review with a patients' focus group, one-on-one interactions with experts and patients, and discussions with stakeholder committee members. Following the patient's focus group, ranking exercises were conducted. A consensus about the attributes and levels was researched during discussions with committee members.

Results. The literature review yielded 40 attributes to consider in patients' treatment choice. During the focus group, one additional attribute emerged. The ranking exercises allowed selecting eight attributes for the DCE. These eight attributes and their levels

were discussed and validated by the committee members who helped reframe two levels in one of the attributes and delete one attribute. The final seven attributes were: treatment modality, pain reduction, onset of treatment efficacy, duration of efficacy, difficulty in daily living activities, sleep problem, and knowledge about their body and pain.

Conclusions. This study is one of the few to comprehensively describe the selection process of attributes and levels for a DCE. This may help ensure transparency and judge the quality of the decision-making process. In the context of a HB-HTA unit, this strengthens the legitimacy to perform a DCE to better inform decision-makers in a patient-centered care approach.

OP33 Treatment Of Mitral Insufficiency And Multicriteria Decision-Making

François Désy, Mireille Goetghebeur, Maria Vutcovici Nicolae, Laurie Lambert (laurie.lambert@inesss.qc.ca) and Michèle de Guise

Introduction. Controversy regarding the efficacy of transcatheter mitral valve repair with a clip (TMVRC) in reducing the mitral regurgitation is related to the lack of solid scientific evidence. Worldwide, refusal or conditional acceptance for implementation of TMVRC, reflect ongoing uncertainty. We sought to apply a systematic multicriteria framework to ensure a fair and reasonable decision regarding the use of TMVRC in Quebec.

Methods. The framework included the following domains: context, quality of evidence concerning safety, efficacy and effectiveness, unmet patient needs, expected volume of patients, and impact on the health system including costs. Each domain within the framework was examined by a review of the literature and through consultations with a scientific advisory committee, a TMVRC clinical expert committee, TMVRC clinical teams, industry representatives and the Institut national d'excellence en santé et en services sociaux (INESSS) clinical excellence committee.

Results. The literature review indicated that uncertainty about the efficacy and effectiveness of TMVRC persists, particularly in the real world context, and this view was supported by scientific experts. The TMVRC clinical teams provided insight into the burden of mitral insufficiency on patients and the health system and their belief in the promise of TMVRC. They also highlighted the challenges of patient selection and organizational issues related to the introduction of TMVRC within their institutions. The advisory committee stressed the need for further evaluation prior to wide diffusion.

Conclusions. Using a multicriteria framework facilitated a more standardized and transparent approach to our literature review and consultations as well as to the development of the proposed recommendations. This was especially important in the context of an evaluation of a promising new approach to treat mitral valve disease with many important uncertainties. This multicriteria approach will facilitate a more standardized process for deliberation on how new health technologies should be implemented into the Quebec health system.

OP34 One-Way Sensitivity Analysis For Cost Effectiveness Analysis

Christopher McCabe, Isaac Awotwe, Mike Paulden, Andrew Sutton (asutton@ihe.ca) and Peter Hall

Introduction. Although stochastic analysis has become the accepted standard for decision analytic cost effectiveness models, deterministic one-way sensitivity analysis continues to be used to meet the needs of decision makers to understand the impact that changing the value taken by one specific parameter has on the results of the analysis. However, there are a number of problems with this approach.

Methods. We review the reasons why deterministic one-way sensitivity analysis will provide decision makers with biased and incomplete information. We then describe a new method - stochastic one-way sensitivity analysis (SOWSA), and apply this to a previously published cost effectiveness analysis, to produce a stochastic tornado diagram and conditional incremental net benefit curve. We then discuss how these outputs should be interpreted and the potential barriers to the implementation of SOWSA.

Results. The results illustrate the shortcomings of the current approaches to deterministic one-way sensitivity analysis. For SOWSA, the expected costs and outcomes are captured, along with the sampled value of the parameter and these are linked to the probability that the parameter takes that value - which can be read off the probability distribution for the parameter used in the stochastic analysis. From these results it is possible to gain insights into probability that a parameter will take a value that will change a decision.

Conclusions. Although a well-used technique, one-way deterministic sensitivity analysis has a number of shortcomings that may contribute to incorrect conclusions being drawn about the importance of certain parameter values on model results. By providing fuller information on uncertainty in model results, it is hoped that the methods here will lead to more informed decision making. Although, as with all developments in the presentation of analytic results to decision makers, care will be required to ensure that the decision makers understand the information provided to them.

OP37 Impact On Uncertainty Of Disaggregating Cost Data

Conor Teljeur (cteljeur@hiqa.ie), Paul Carty and Máirín Ryan

Introduction. Economic models contain several parameters ordinarily subject to uncertainty. Unlike most other model parameters, costs can constitute numerous distinct components. For example, a surgical intervention can require a variety of disposable and reusable equipment. A micro-costing output may be disaggregated or presented as a total cost. Uncertainty could be applied to individual cost components or to total cost. We aimed to explore how disaggregation of cost data may impact on uncertainty using a case study.