OP88 Drawing Lines In The Sand: How Do We Define The Scope Of Analysis In HTA And Economic Evaluation?

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Introduction. We explore how the scope of analysis is defined in health technology assessment (HTA) and economic evaluation and consider the potential implications of these decisions.
Methods. The scope of analysis, including decisions about which methods and domains of HTA to include in the assessment, which costs, and health outcomes are most meaningful, and which comparators are the most relevant are typically informed by the needs of the decision-maker. We undertook two systematic scoping reviews to assess: (i) to what extent systems thinking is considered in literature-based technology assessments; and (ii) how the scope of the analysis is defined in economic evaluation using *Clostridioides difficile* infection as an exemplar. We synthesized the findings from these reviews and offer three key observations for future research and exploration in the field of HTA.
Results. Our scoping reviews found that the scope of analysis in economic evaluations typically focus on single interventions, often ignoring upstream and downstream interventions. Similarly, published technology assessments have narrowly defined and inconsistent scopes of analysis, with limited consideration of indirect health and non-health impacts. Three key observations for the field of HTA include: (i) economic evaluations focus on the value of single heath interventions. A focus on a single health intervention may simplify the analysis; however, will this siloed decision-making lead to optimal health resource allocation? (ii) published assessments have inconsistently defined scopes of analysis. A decision problem that focuses on the needs of the decision-maker is practical; however, will inconsistencies in perspectives across assessments create unfair conceptualizations of value? (iii) HTA is technology-focused, not patient-focused. A technology-focused HTA system aligns with the technology diffusion process; however, does this move us away from the patient-centered mandate of HTA?
Conclusions. The dynamic nature of HTA leads to many conceptualizations of value. Considering the potential implications of narrowly defined, inconsistent, and technology-focused scopes of analyses may have consequences on achieving a patient-centered high-quality health system.

OP89 The CE-Signal, A New Simplified Health Technology Assessment Method To Determine Whether Interventions Are Cost-Effective

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Introduction. Conducting a cost-effectiveness analysis (CEA) is resource consuming, and therefore the Dutch National Health Care Institute (ZIN) only performs those for interventions with a high budget impact. Sometimes, cost-effectiveness (CE) estimates are clearly below or far above reference values, which makes full cost-effectiveness assessments less vital. The objective of this study was to develop an efficient and simplified method to identify interventions that are clearly cost-(in)effective.
Methods. The method makes use of headroom analysis. Several HTA experts and other relevant stakeholders have been asked to provide feedback on a preliminary version of the CE signal.
Results. The method consists of five steps. In the first step (i) the relevant willingness-to-pay threshold is determined. Reference values are used by ZIN for the maximum willingness-to-pay per incremental quality-adjusted life year (QALY), depending on burden of disease. In next step (ii) the health gain that can realistically be obtained with the new treatment is estimated. Hereby the effect of the intervention on the clinical outcomes, quality of life and gained life years is determined to estimate the number of QALYs gained, including uncertainty. Then (iii) the societal cost maximum (SCM) of the new treatment is calculated by multiplying step 2 with step 1. In step four (iv) the incremental treatment costs are estimated looking at both the costs and savings for both treatments options for the average patient. In the final step (v) the incremental treatment costs are compared to the SCM to determine if the intervention is probably cost effective, probably not cost-effective or if a conclusion cannot be drawn.
Conclusions. This method has proven to be feasible and could be a valuable addition to the current cost-effectiveness assessment toolbox. The CE-signal is being validated against performing a full cost-effectiveness analysis.

OP90 Optimizing Health Technology Assessment And Appraisal For Orphan Drug Reimbursement: Experiences And Tools For Improvement

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Introduction. The very high costs of orphan drugs, together with the uncertainties regarding their (cost-)effectiveness raise questions regarding the efficiency and legitimacy of their health technology assessment (HTA) and appraisal process. The aim of the present, qualitative study was to investigate how experts on the reimbursement of these treatments perceive the HTA and appraisal process in their country. Moreover, it aimed to provide specific conditions and practical recommendations for their improvement.
Methods. Twenty-two European experts from 19 different countries were included in a qualitative survey and semi-structured interviews. Transcripts were analyzed using the qualitative data analysis software Nvivo. A grounded theory approach was adopted to develop a set of well-defined concepts from the cyclic analysis of the empirical data.
Results. First, analysis of the expert interviews yielded five good practices for an efficient HTA and appraisal of orphan drugs: a high level of transparency, patient involvement, a clear decision-making structure with room for flexibility, mechanisms to minimize bias and an explicit consideration of the opportunity cost. Meanwhile, participants highlighted several barriers to the overall process, such as a lack of trust between the different stakeholders and imbalances in negotiation power. In addition, the results allowed to identify a number of 'contextual' determinants that may undermine the legitimacy of the final decision, such as bias and the perverse effects of the orphan drug legislation. Drawing from the experts' experiences, a toolkit was developed that includes an extensive number of specific recommendations (and conditions) for decision-makers to improve the legitimacy and efficiency of their HTA and appraisal of orphan drugs.
Conclusions. Overall, the results showed that decision-makers should focus on limiting the impact of the contextual determinants rather than improving the methods included in the HTA. This will contribute to further legitimize reimbursement choices for orphan drugs towards the wider public.

OP91 The Current State Of Disease-Specific Registries For The Monitoring Of Expensive Pharmaceuticals In The Netherlands

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Introduction. On a regular basis, new and innovative medicines come to the market; with uncertainties surrounding their exact effect in patients. To address these uncertainties, disease-specific registries are commonly being used. The Dutch National Health Care Institute (Zorginstituut Netherland [ZIN]) started, in collaboration with stakeholders, a national program on the management and coordination of disease-specific registries. The main goal is to improve the quality and consistency of these registries and thereby increase the value of the data in monitoring innovative, expensive medicines. As a starting point for this program, we performed a study on the current state of these registries in the Netherlands.
Methods. Using an initial list of 114 registries, we sent out questionnaires to 58 disease-specific registries that collected information on medicine use. Thirty registries responded and their registries were used for the analysis.
Results. Of 30 registries that responded, 15 registries collect information on orphan medicinal products. Most registries are in the field of internal medicine (n = 8), oncology (n = 6), and rheumatology (n = 5). The size of the registries ranged between 250 patients (orphan diseases) and more than 10,000 patients (oncology). Only 13 registries collect information on patient reported outcomes. Data collection is mostly performed manually and standard coding systems such as Systemized Nomenclature of Medicine (SNOMED), Logical Observation Identifiers, Names, and Codes (LOINC), and The International Classification of Diseases 10th Revision (ICD-10) are not routinely used. Finally, our results show that most registries are (partly) dependent on the funding of pharmaceutical companies.
Conclusions. Our assessment shows variation in the type, goal of, governance, and funding of the disease-specific registries investigated. We believe that these results show the importance of further national coordination of the disease-specific registries to increase the usability of their data to address the uncertainties surrounding these innovative, expensive medicines.

OP92 Impact Of Real-World Evidence On Health Technology Assessment And National Guidance For Interventional Procedures: A UK Perspective

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Introduction. The National Institute for Health and Care Excellence (NICE) interventional procedures programme (IPP) issues guidance on the safety and efficacy of new interventional procedures (IPS). The IPP considers a range of evidence from randomized controlled trials (RCTs) to case series to make final recommendations. Real-world evidence (RWE) can provide additional information on long-term outcomes and patient population characteristics that are not easily captured by RCTs. This study explores the impact of RWE in complementing RCTs on long-term safety and efficacy for national guidance development.
Methods. We review the impact of RWE in IPS guidance (IPG) updates and change in guidance recommendations in the last 5 years. This is done by analyzing NICE guidance updates and supporting evidence. A range of RWE was considered in the supporting evidence, for example, registries and clinical audits.
Results. The addition of RWE evidence to IPP guidance update has led to significant changes in the recommendations. For example, in one recent IPG, standard arrangements recommendation based on

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