

The deliberative dialogue allowed stakeholders—represented by people with disabilities, policymakers, decision-makers, health professionals, members of associations, and researchers—to actively engage in constructing the synthesis.

Conclusions: The stakeholder engagement concluded that the project promoted social inclusion and equal, universal, and comprehensive access to social rights by people with disabilities. The experiences of stakeholders in society were incorporated into public policy and guided decision-making in health and social care.

PD184 Health Technology Assessment (HTA) Topics That Respond To National Needs: Considerations Around The Topic Selection Process When Institutionalizing HTA

Elizabeth Peacocke, Lieke Heupink,
Aparna Ananthkrishnan and
Katrine Frønsdal (Katrine.Fronsdal@fhi.no)

Introduction: Most HTA processes follow similar institutional mechanisms, starting with topic selection and prioritization, followed by analysis (appraisal, deliberation, and decision-making) and implementation. The process of conducting an HTA is financially and time intensive. Therefore, to sustain HTA decision-makers, especially in countries with limited capacity, selecting topics for HTA that most respond to national needs is crucial.

Methods: Information on topic identification, selection, and prioritization (TISP) processes was taken from a recent report published by the Norwegian Institute of Public Health (NIPH) on how to support capacity building for HTA in low- and middle-income countries. An unpublished survey of 29 national HTA organizations around the world was also performed by the NIPH asking about their TISP processes. Issues around the institutional and organizational aspects necessary for explicit and transparent TISP processes were identified and discussed through an iterative process.

Results: The comprehensiveness of TISP processes varied according to each country's needs and the types of decisions supported by HTA. Accordingly, the resources available for allocation within the health system, the number of dedicated personnel available to complete HTAs, and the number stakeholders and institutions involved in the decision-making process may all be relevant considerations for TISP. In countries where HTA was well-established, the process for TISP was usually institutionalized or at least somewhat formalized. In settings where HTA was emerging or relatively new, or where there may not be the necessary supporting institutional mechanisms, there was limited normative guidance on how to implement TISP.

Conclusions: When institutionalizing HTA, we argue for including formal and explicit processes for the topic selection step that include: (i) a clear link to health system feasibility; (ii) process transparency to

ensure legitimacy and impact; and (iii) patient and public engagement. Insights and experiences from countries with more formalized HTA systems can provide valuable lessons.

PD185 Is The Incorporation Of Medications For Ultrarare Diseases In Brazil Predominantly Driven By Costs? A Shift In Paradigm

Daniel da Silva Pereira Curado,
Juliana Machado-Rugolo (jr.machado@unesp.br),
Denis Satoshi Komoda,
Monica Aparecida de Paula de Sordi,
Juliana Tereza Coneglian de Almeida, Alan Fonseca,
Silvana Molina Lima, Silke Anna Theresa Weber,
Luis Gustavo Modelli de Andrade, Jean-Eric Tarride,
Lehana Thabane,
Marilia Mastrocolla de Almeida Cardoso and
Marisa Santos

Introduction: Incorporating technologies for ultrarare diseases (URD) poses challenges for global health technology assessment (HTA) agencies. Difficulties include defining an analytical framework and establishing differentiated cost-effectiveness thresholds. The rise of technological innovations intensifies demands from healthcare professionals, media, and the general population, placing pressure on healthcare systems in developing countries.

Methods: To analyze ultrarare medicine costs in submissions to the Brazilian National Committee for Health Technology Incorporation (CONITEC), data from HTA reports on URD (from 2012 to 2022) were extracted. Diseases were classified as URD based on an epidemiological criterion or Orphanet consultation (prevalence ≤ 1 per 50,000 inhabitants). Extracted variables included initial and final prices, annual patient cost, incremental cost-effectiveness ratio (ICER), and initial and final CONITEC recommendations. Price differences were calculated by the Brazilian Medicines Market Regulation Chamber.

Results: Among 53 reports, 30 featured economic evaluations, with only 13.3 percent initially receiving positive recommendations. However, eight gained favor, including post-consultation, price-conditioned, and risk sharing-based approvals. Annual medication costs ranged from USD17,439.20 to USD1,108,237.00 per patient, with discounts of between 25 and 64 percent. Despite some technologies having ICERs that were significantly higher than the national threshold, no statistical relationship was found between ICERs and recommendations. Monthly and annual costs varied, with higher costs for heterogeneous diseases and lower costs for metabolic conditions. Sensitivity analyses, using both deterministic and probabilistic analyses, were conducted in 58 percent of the reports.