PD240 Report Of Outcomes In Health Technology Assessment For Technologies In Ultrarare Diseases In The Brazilian Public Health System

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Introduction: Ultrarare diseases (URD) represent a challenge to health technology assessment (HTA). The traditional framework for assessing efficacy and cost effectiveness may be biased to include clinically relevant outcomes, leaving patient-centered outcomes doomed to neglect. Here we explore patient-centered outcomes in the context of patient and citizen involvement in the assessment of URD by the Brazilian National Committee for Health Technology Incorporation (CONITEC).
Methods: We assessed 53 HTA reports from CONITEC that evaluated URD-related technologies (and included highlights of patients' and citizens' perspectives during recommendation meetings) published from 2012 to 2022. Data extraction was performed by two independent researchers. Data on year of report, sex, ethnicity, category (patient or family), and previous experience with the assessed technology were extracted and analyzed using descriptive statistics. Patients' and citizens' narratives were collated from the reports. A thematic analysis was conducted according to patient-centered outcomes and technology-related outcomes and was then compared with the evidence synthesis protocol described in the HTA.
Results: Only seven URD-related HTA reports registered patient or citizen participation, all of which were published in 2022. The age of two participants was reported (both 17 years). Six participants were women. Ethnicity was not reported. All participants had previous experience with the technology. Four participants were family or caregivers and three were patients. Considering patient-centered outcomes, physical (muscular strength) and emotional (self-confidence) improvements that positively affected independence in basic daily functions were reported. These functions included activities such as dressing, self-care, cooking, and leisure. Advantages listed for the assessed technologies included the possibility of self-administration of medication (e.g., swallowing a pill, opening a medicine bottle, and using a syringe).
Conclusions: The results show that although, in some cases, primary outcomes reported in evidence synthesis protocols include patient-centered outcomes (e.g., activities of daily living), in other cases the evidence synthesis failed to identify relevant studies. In other cases, the reports failed to differentiate between primary and secondary outcomes or to fully account for patient-centered outcomes.

PD241 Supporting Patient Groups To Enhance Their Input To Health Technology Assessment

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Introduction: The Scottish Medicines Consortium (SMC) patient group submission process includes a written submission of patient experience evidence from Patient Group Partners (PGPs). To maximize the relevance and quality of information included in submissions, the first draft is reviewed by the Public Involvement Team and feedback is provided. The submission is then amended by the submitting PGP before final submission.
Methods: Upon receiving a new written patient group submission, the Public Involvement Team reviews it and provides feedback highlighting any evidence gaps and areas where wording could be amended to strengthen the patient voice. The draft submission is then amended by the submitting PGP, with support from the SMC reviewer. Once finalized, the submission is collated into the body of evidence used by the SMC Committee members to assess a new medicine. The satisfaction of participating PGPs is continually assessed by an online survey. During the 2021 to 2023 period, the SMC received 232 patient group submissions, with 77 percent being amended and strengthened after receiving feedback.
Results: The feedback and collaboration on amendments to draft submissions has improved the quality of patient experience evidence submitted to the SMC by PGPs. PGPs value their draft evidence being reviewed by the SMC Public Involvement Team prior to final submission. This approach resulted in high levels of trust in and satisfaction with how the SMC involves patient representatives in medicine assessments. In 2022, 89 percent of the 28 PGPs surveyed were very satisfied and 11 percent were satisfied with the support provided during the submission process.
Conclusions: Evaluation of the SMC's formal process to provide input and feedback on draft written patient group submissions demonstrates high levels of satisfaction with how the SMC works in partnership with PGPs. This approach has strengthened written evidence of patient experiences for SMC assessments. Furthermore, it helps to build and maintain a partnership approach in how the SMC works with PGPs.

https://doi.org/10.1017/S0266462324004586