OP58 Challenges In Maintaining Up To Date Health Technology Assessments in Rare Diseases: Lessons From Fabry Disease In Australia

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Introduction: Fabry disease is a rare, inherited X-linked lysosomal storage disease characterized by a wide spectrum of heterogeneously progressive clinical phenotypes, and which results in progressive kidney disease, cardiomyopathy, cerebrovascular disease, and reduced life expectancy. Disease-specific therapy aims to improve symptoms, stabilize current disease and delay progression. In Australia treatment access requires that patients meet pre-specified criteria, which have been in place for more than 15 years. Patient questions prompted the patient organization, Fabry Australia, to investigate why these criteria had remained unchanged despite significant progress in the understanding and management of Fabry disease.
Methods: A panel comprising two members of Fabry Australia and its Medical Advisory Committee conducted a review of the literature. The aim of this was to inform the clinical quality of the Australian treatment access criteria with reference to international guidelines and contemporary data. The findings from the literature were applied to develop consensus recommendations for classification and Fabry-specific treatment initiation criteria in diagnosed patients.
Results: Evidence supports earlier treatment with reduced barriers to access in some circumstances. Australian access criteria are misaligned with this. They do not distinguish between classical and non-classical Fabry phenotypes, neglect the impact of quality of life and gastrointestinal symptoms, and impose symptom-severity related criteria, which may lead to unnecessary treatment initiation delay. An updated framework is presented. It differentiates phenotypes, facilitates more timely access to Fabry-specific treatment for classical males, and supports relevant organ involvement criteria in classical females and patients with non-classical disease.
Conclusions: A well-performing health technology assessment system facilitates patient access to cost-effective treatments that improve health outcomes. Timely treatment initiation is important to avoid irreversible organ damage in Fabry patients. Patients' questions about out-dated access criteria has prompted research and uncovered barriers that are no longer clinically valid. The perspectives of the patient as a stakeholder in their disease management should not be overlooked when assessing the value of health technologies in the rare disease setting.

OP59 Do Digital Health Terms Provide Sufficient Information To Allow For Health Technology Assessment?

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Introduction: Umbrella digital health term (DHT) (digital health, eHealth, mHealth, telehealth, and telemedicine) definitions contain insufficient information about patient value for health economics and outcomes research and health technology assessment (HTA) purposes. Qualitative content analysis of secondary DHT (e.g., telesurgery and teleradiology) definitions was performed by the ISPOR Digital Health Special Interest Group to determine if they were more useful for health economics and outcomes research purposes.
Methods: Secondary DHT definitions were extracted from a previous scoping review and consolidated by reviewer pairs using uniform rules. Definitions were analyzed for explicit (directly stated) or implicit (inferred) information on 24 categories: Patient, Intervention, Comparator, Outcome, Timing, Setting (PICOTS); the Shannon-Weaver communication model (SWE) (sender, message, encoder, channel, decoder, and receiver, extended with mode of information exchange); the quality domains of Agency for Healthcare Research and Quality (AHRQ) (safe, effective, patient-centered, timely, efficient, and equitable); information related to applied technology or geographic scope; and the World Health Organization (WHO) classification of digital health interventions v1.0 (digital health interventions category, health system challenges, and system categories).
Results: Across 107 unique definitions of 73 secondary DHTs, the number of explicitly or implicitly addressed categories across the frameworks ranged from zero to 15, with references to elements of PICOTS (79.4%), SWE (90.7%), AHRQ (30.8%), applied technology (52.3%), geographic scope (0%), and WHO frameworks (86.9%). PICOTS information was found for Patients in 35 percent of definitions, Intervention in 59 percent, Comparator in 20 percent, and Outcomes in 18 percent.
Conclusions: Secondary DHT definitions do not adequately specify PICOTS or other characteristics of interest for HTA. An online Delphi survey has been launched among a wider group of ISPOR members to identify the minimum information set to define patient facing DHTs for evidence summaries and value assessments. The results of this research should be shared for discussion with other digital health stakeholder groups.

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