A MULTICRITERIA DECISION ANALYSIS PROPOSAL OF EVALUATION CRITERIA FOR TREATMENTS OF RARE DISEASES IN BRAZIL

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BACKGROUND

Multiple Criteria Decision Analysis - MCDA is a tool that allows a systematic and explicit consideration of multiple factors that influence the decision, through identified criteria, called a framework, in a manner in which a weight is assigned to each criterion and thus make their values and goals clear. (1) The use of this methodology in health technology assessment processes promotes the assessment of complex problems based on a comprehensive set of criteria, organizing a discussion among interested parties, and providing everyone with access to the same set of information. This structural proposal has as its main objective to open space for dialogue among market participants, presenting an initial path for a specific modality of evaluation of technologies for the treatment of rare diseases in Brazil.

OBJECTIVES

From a multi-stakeholder perspective, propose relevant criteria for the evaluation of rare disease treatments in Brazil.

METHODS

The Multicriteria Decision Analysis (MCDA) exercise was developed based on the ISPOR GRP on MCDA and a literature review process to evaluate the already published MCDA frameworks for rare diseases and orphan drugs. For this, a research was carried out in the PubMed database on September 19, 2022, based on the search strategy described by Andrés-Nogales et al. 2021 (2), with the following search key: "(evidem OR MCDA OR multicriteria decision analysis or discrete choice OR conjoint analysis OR hierarchic analytic) AND (economic OR reimbursement OR financing OR finance OR pricing) AND (rare disease OR orphan drug OR orphan)".

Inclusion criteria: articles that present MCDA framework proposals for products aimed at rare diseases.
Exclusion criteria: letters, editorials, and MCDA processes for non-rare diseases were excluded.

Multicriteria decision analysis – Suggested domains and criteria's for evaluating treatments for rare diseases in Brazil

Domain	Criteria
Disease	Affected population
	Disease severity
	Impact on Patient's Physical Health
	Impact on the Patient's Psychological Health
	Impact on Patient Independence Level
	Impact on daily activities
	Economic burden of disease
	Impact on patient life expectancy
	Impact on the patient's quality of life
Comparative results / Intervention Value	Comparative Effectiveness
	Comparative safety/tolerability
	Patient perception of the new treatment results
	Coverage of currently unmet therapeutic needs
	Uncertainty about proving the new treatment's effectiveness
	The patient's reported health-related quality of life with the new treatment
	Social impact of new treatment
Economic Aspects	Annual cost of new treatment
	Budget Impact
	Cost-effectiveness
	Management of patients who are eligible for the new treatment.
	Value-based agreement proposal
	Other countries' HTA bodies have recommended the new treatment.
	Logistics needs for the health system to absorb the new technology

RESULTS

Sixteen publications were chosen from a pool of 52. For the development of the MCDA proposal, Brazilian laws, health system regulations, and the HTA official process were also considered. This proposal established 5 domains (disease, comparative intervention results, therapeutic value, economic aspects, and health technology evaluation analysis) and correspondent criteria: Affected population; disease severity; Patient's social life impact; Economic load; Caregiver's QoL impact; Life expectancy; Morbidity; Vulnerable groups; Coverage of unmet medical needs; Comparative effectiveness; Comparative safety/tolerability; Comparative results perceived by the patient; Level of evidence; BIA: CEA/CUA; Logistics needs; HTA recommendations in other countries.

CONCLUSION

The establishment of a multi-criteria process would bring greater transparency and assertiveness to the evaluation processes of treatments for rare diseases. Furthermore, the evaluation of its effect on patient and caregiver HRQL, the new treatment therapeutic benefit extent, availability and efficacy of other therapeutic options and severity of the disease should influence the decision-making process in a more even-handed manner.

REFERENCES

1. World Health Organization. Access to new medicines in Europe: technical review of policy initiatives and opportunities for collaboration and research. 2015.
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