

Multicriteria Decision Analysis: A Decisionmaking Tool for Clinicians?

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Background & Objective

- Healthcare decisionmaking is a complex process that requires consideration of a wide range of scientific and contextual criteria, and inherently involves value judgments.¹
- At the policy level, this process demands transparency, consistency, and accountability to be perceived as legitimate and to increase the likelihood of making good decisions.^{2,3} At the clinical level, development of recommendations to guide clinical practice also requires consideration of a broad range of aspects to ensure optimal care^{4,5} and social responsibility.
- Clinicians are faced with a constantly increasing medical literature.^{6,7} Many obstacles impede their search of information (e.g., lack of time, resources poorly organized or difficult to access, etc.).⁸ Short summaries of up-to-date, high-quality evidence, web-based resources, and more comprehensive and systematic monitoring efforts are needed to keep abreast of current literature, and support patient care.⁶⁻⁸
- EVIDEM is an MCDA-based adaptable framework to synthesize and consider evidence for each decision criterion.^{9,10} It provides a consistent structure to organize evidence and facilitate both clinical and policy decisionmaking.
- Prader-Willi syndrome (PWS) is a rare and complex multisystem disorder with serious long-term consequences. Use and coverage of growth hormone (GH) in patients with PWS vary widely not just pointing to a need to clarify its benefits.
- Objective:** To adapt and apply an MCDA-based framework to support clinical decisionmaking and the development of clinical practice guideline (CPG) using GH for PWS patients as a case study.

Methodology

- An extensive review of the literature was performed to synthesize evidence for each criterion of the EVIDEM framework composed of:
 - a quantitative Core MCDA Model including 13 universal normative decision criteria organized in six domains (Disease impact, Context of intervention, Intervention outcomes, Type of benefit, Economics, and Quality of evidence);
 - a qualitative Contextualization Tool including six contextual criteria organized in two clusters (Ethical framework; Overall context).
- The framework was adapted to include specific outcomes measures of GH treatment in PWS patients by expanding the Efficacy criteria into six subcriteria.
- Data was synthesized at four levels of detail and validated by experts using an interactive web system.
- During a consensus workshop held to develop international CPG for GH therapy for patients with PWS, 28 experts (clinicians, patient representative, ethicist, methodologists & researchers) field-tested the EVIDEM framework.
- To capture individual perspectives on the relative importance of criteria, participants assigned weights to the criteria of the MCDA Core Model on a scale from 1 to 5 and assigned weights to the Efficacy subcriteria using a point allocation technique;¹¹ they were also asked to indicate whether contextual criteria should be systematically considered.
- To assess the performance of GH for patients with PWS, participants scored quantitative criteria and subcriteria on a 4-point scale (0,1,2,3) and assigned impact (negative, neutral, positive) to contextual criteria; synthesized evidence provided for each criterion supported this process.
- An MCDA value estimate was obtained using a linear model combining normalized weights and scores.
- At the end of the exercise, participants completed a survey to collect feedback on the framework and process.

Results

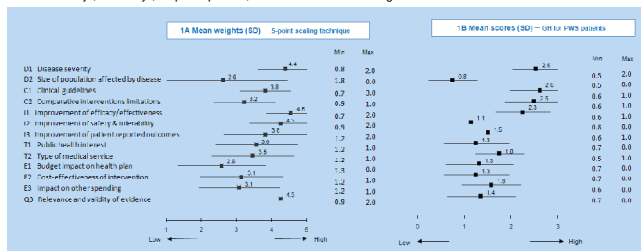
1 MCDA Core Model – estimating value based on universal criteria

1A Individual perspectives on criteria captured by weights – 5-point scaling technique

- Importance of criteria (independent of intervention) varied widely among participants; most important criteria were "Efficacy/effectiveness", "Disease severity", "Safety and tolerability", and "Quality of evidence" while "Size of population" and "Budget impact" were least important, reflecting an emphasis on clinical aspects.

1B Performance of GH for patients with PWS

- Highest scores were for "Clinical guidelines", "Disease severity", and "Limitations of other interventions", reflecting how GH fulfills an unmet need for a very severe disease. Efficacy scored fairly high while quality of evidence was low. The safety criterion received one of the lowest scores and had the largest variation among participants (SD:0.8), reflecting safety issues.
- The MCDA value estimate of GH for PWS (combining normalized weights and scores) was 57% of maximum value with "disease severity", "efficacy", "expert opinion", and "unmet needs" being the main contributors to value.



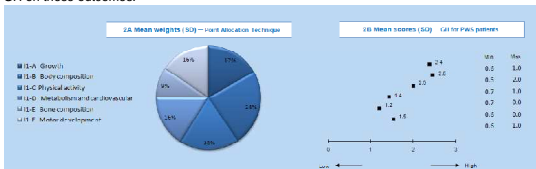
2 Efficacy subcriteria – identifying outcomes of interest

2A Individual perspectives on subcriteria captured by weights – point allocation technique

- The highest weights were assigned to the subcriteria "body composition" (24% of points) and "physical activity" (18% of points).
- Weight attributed to subcriterion "Growth" was only 17% of points; 46% of respondents attributed $\leq 10\%$ of points to "Growth".

2B Performance of GH for patients with PWS on efficacy subcriteria

- The highest scores for GH in PWS patients were given to "Growth" and "Body composition".
- Scores for "metabolism and cardiovascular", "bone composition" and "motor development" were low reflecting a limited efficacy of GH on these outcomes.



3 Contextualization tool - qualitative impact of contextual criteria

3A Individual perspectives on criteria

- Most participants felt that the 3 criteria of the ethical framework (Et1-3) should be considered systematically, independently of the intervention under scrutiny.

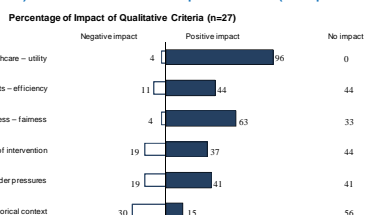
3B Impact of contextual criteria on appraisal of GH for PWS patients

- A majority of participants indicated that consideration of utility and fairness had a positive impact on appraisal of GH for patients with PWS; for the other criteria, opinions were divided.

3A Consideration of criteria (% responders)

Criterion should be considered (%)	Et1: Goals of healthcare – utility	Et2: Opportunity costs – efficiency	Et3: Population priority & access – fairness
96	96	85	85
93	93	42	26
42	19	19	19
26	30	15	56

3B Qualitative impact of criteria (% responders)



Discussion and Conclusion

This MCDA-based approach allowed us to capture perspectives and appraisals at the individual level and to identify criteria contributing to the value of GH for patients with PWS which are disease severity, efficacy, expert opinion, and unmet needs.

The relatively low importance given to population size and to budget impact of therapeutic interventions may reflect consideration given to the intervention paradigm chosen for this study, namely, a rare genetic condition with a costly therapy.

Adaptation to include efficacy subcriteria allowed us to identify the outcomes of most interest to participants, namely body composition and physical activity. This is discrepant with the rationale on which the indication of GH for PWS was granted by licensing bodies, (namely on growth and body composition).

In this adaptation of the framework, two weight elicitation techniques were used conjointly, indicative of the flexibility of the framework.

The majority of participants (70%) found an interest in the MCDA exercise, either for stimulating reflection or facilitating discussion and deliberation suggesting that MCDA-based approaches can be useful decisionmaking tools for clinicians.

Inclusion of subcriteria allowed us to refine the reflection and to adapt the framework to specific decisionmaking applications, which will be further explored for the policy application in this research project.

This comprehensive by-criterion approach provides a common road map to streamline clinical and policy decisionmaking to optimize patient health, resource allocation and healthcare system sustainability.

The ultimate goal is to bridge the gap between researchers, HTA, policy decisionmaking, clinical practice and patient concerns.

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