

# MULTICRITERIA DECISION ANALYSIS FOR HEALTH CARE DECISION MAKING IN CATALONIA: DEVELOPMENT OF A SPECIFIC EVALUATION FRAMEWORK FOR ORPHAN DRUGS

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## BACKGROUND & OBJECTIVES

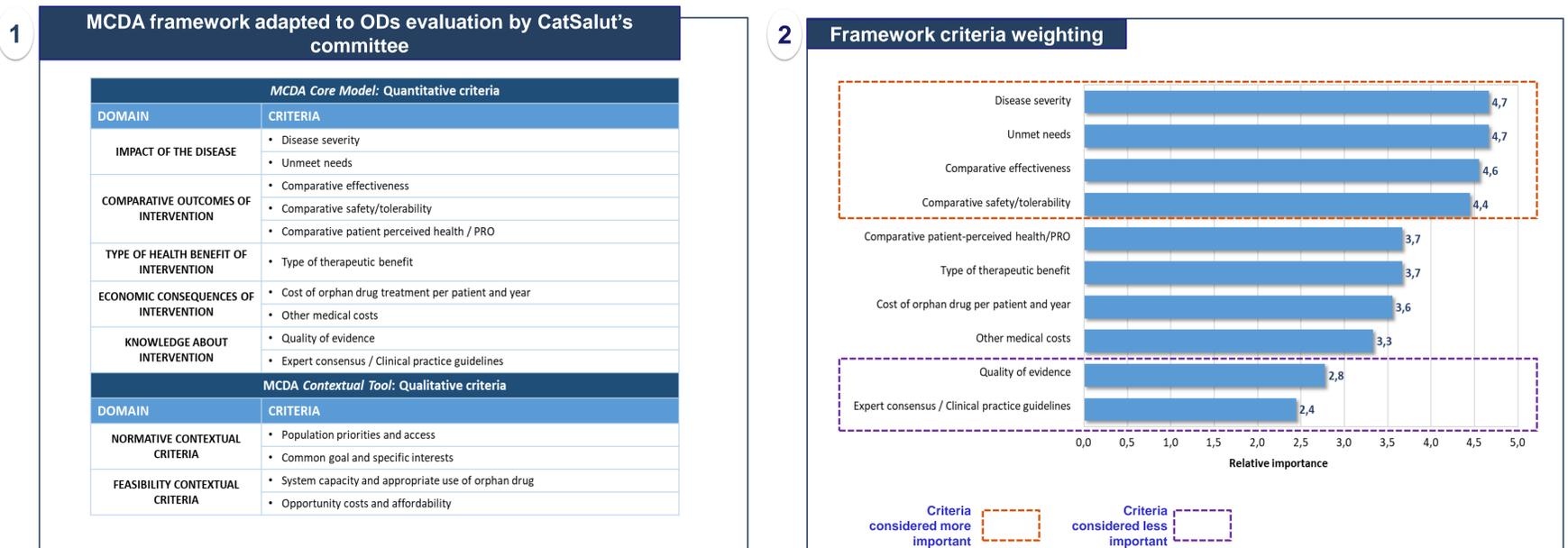
- Decision making processes in a healthcare institution require a standardised procedure. This procedure requires transparency, consistency, and accountability to be perceived as legitimate by the public and health care providers to increase the likelihood of making right decisions.
- Decision-making on access to orphan drugs (ODs) faces special challenges due to often scarce evidence, i.e: uncontrolled and small trials, lack of a comparator treatment, and/or high prices. Thus, the evaluation of ODs often is supported by additional criteria such as unmet needs, disease severity or patient's quality of life, among others, which may be difficult to systematize.
- The objective of this study was to validate the Multi-Criteria Decision Analysis (MCDA) framework specific for Orphan Drugs (ODs) developed in a previous pilot study in Catalonia, aimed to facilitate the discussion and homogenize the assessment of ODs by the decision-making committee of the Catalan Health Service (CatSalut).

## METHODS

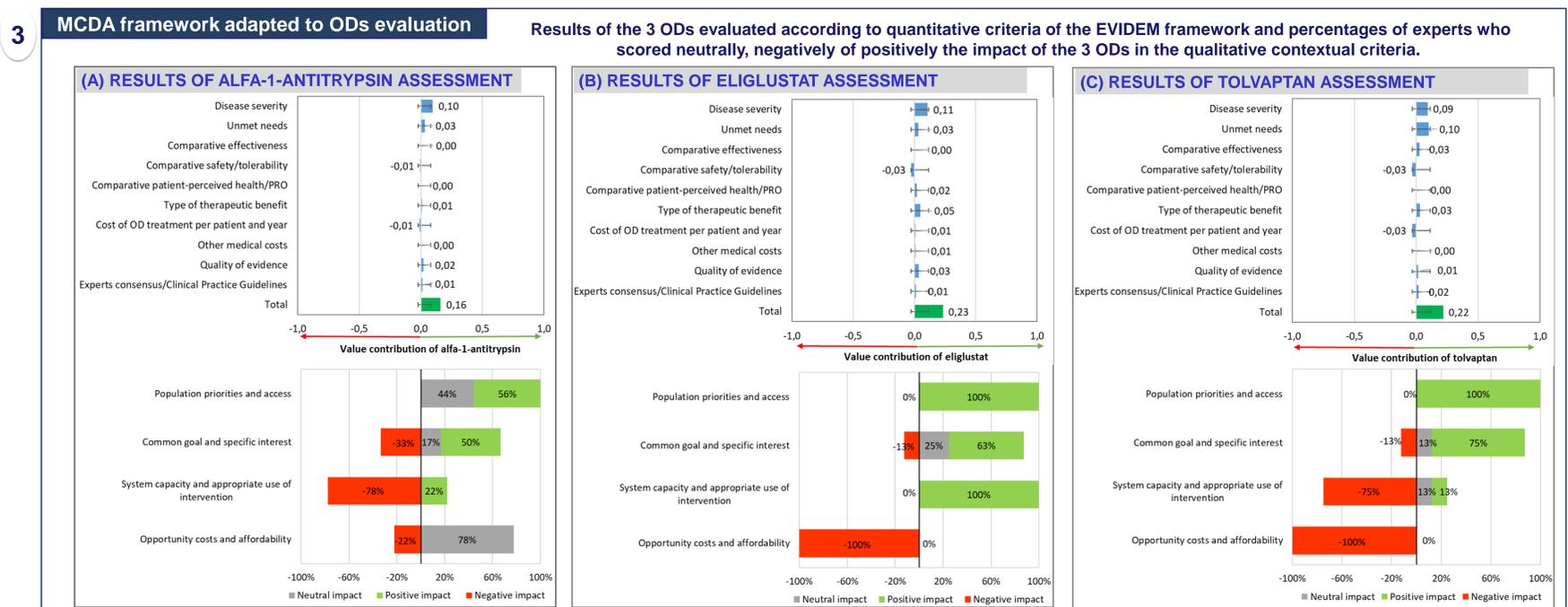
- A preliminary framework based on EVIDEM (v.4.0)<sup>1</sup> and adapted according to a literature review<sup>2</sup> was developed for its use in ODs evaluation and was validated by representatives of the decision-making committee of CatSalut<sup>3</sup>.
- The final version of the framework was agreed, weighted and tested by the committee members of a decision making committee in 3 ODs (tolvaptan for autosomal dominant polycystic kidney disease, alpha-1-antitrypsin for alpha1-antitrypsin deficiency and eliglustat for Gaucher disease). The committee members (n=9) rated individually the EVIDEM matrix for each drug assessment according to their preferences and afterwards a reflective discussion was conducted amongst all the members.

## RESULTS

- In the validation phase, some criteria were removed ("size of population", "type of preventive benefit", "non-medical costs", "mandate and scope of healthcare system", "environmental impact", "political/historical/cultural context") or adapted ("therapeutic benefit") from the standard framework based on EVIDEM (v.4.0) for CatSalut purposes.
- Figure 1 shows the final version of the MCDA framework adapted to ODs evaluation by CatSalut members and figure 2 shows the weights of the final framework criteria (from 0, less important to 5, most important).



- The assessment of 3 ODs was conducted to rate the evidence matrix. Figure 3 shows the results of the assessment of alpha-1-antitrypsin (A), eliglustat (B) and tolvaptan (C). The reflective discussion was perceived as very relevant to support inputs for health decision-making processes, reflecting drug value, positioning the 3 ODs within the different dimensions and finally discussing the overall value (rescaled to range from 0 to 1) of the drug by CatSalut.



## CONCLUSIONS

A final MCDA framework specific for ODs was developed and validated to assess the value of the ODs evaluated in the health care service. The proof-of-concept using MCDA as the support for evaluation of three ODs demonstrated that MCDA can be considered a useful methodology that facilitates communication between stakeholders, contextualizes the relevant data of each drug and complements the technical evaluation appraisal supporting a more transparent and harmonized decision.

## REFERENCES

- [1] EVIDEM Framework (v 4.0). Access date: May 2017. <https://www.evidem.org/>
- [2] Criterios de financiación y reembolso de los medicamentos huérfanos. ISCIII. Abril 2016. Disponible en: <http://publicaciones.isciii.es>
- [3] Gilabert-Perramon A, *et al.* Drug evaluation and decision making in Catalonia: development and validation of a methodological framework based on multi-criteria decision analysis (MCDA) for orphan drugs. International Journal of Technology Assessment in Health Care. 2017. 33(1), 111-120

