

DEVELOPMENT OF AN EVALUATION FRAMEWORK FOR ORPHAN DRUGS BASED ON MULTI-CRITERIA DECISION ANALYSIS (MCDA) FOR HEALTH CARE DECISION MAKING IN CATALONIA

M. Fontanet¹, A. Prat¹, M. Obach¹, A. Vallano¹, C. Pontes¹, J. Torrent¹, X. Badia², L. Guarga²

¹CatSalut; ²Omakase Consulting S.L.

BACKGROUND & OBJECTIVES

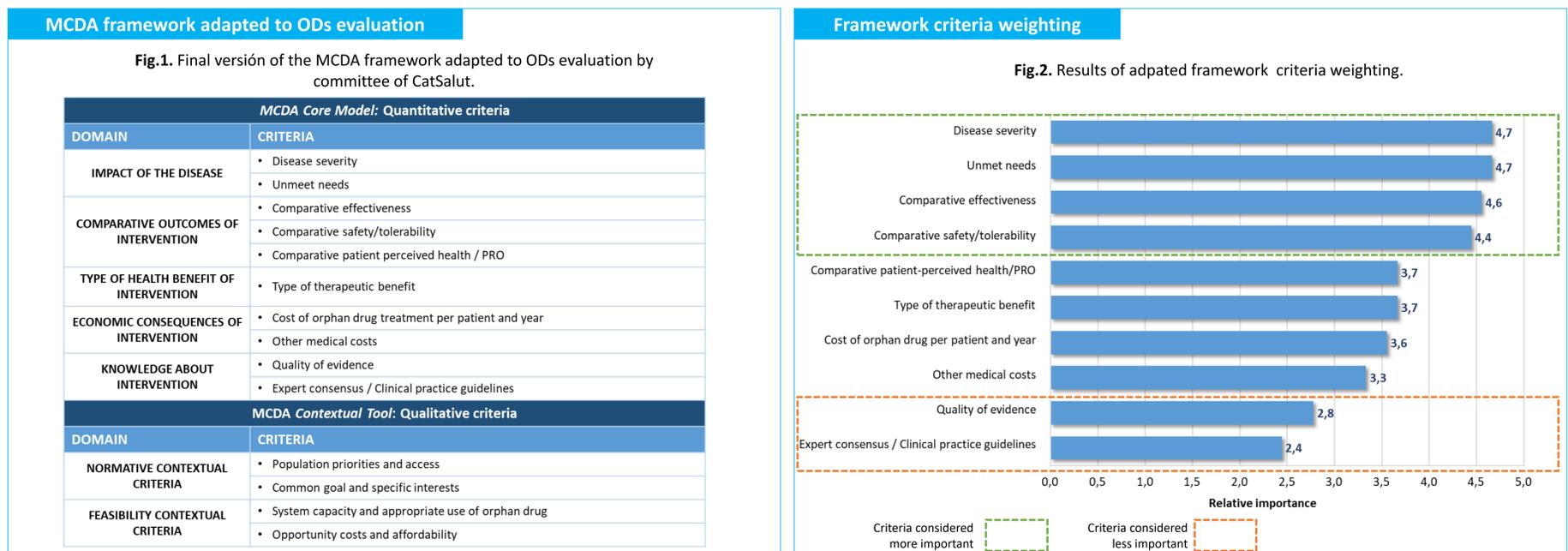
- **Decision making in healthcare** and, specifically, in decision related to **include or not an orphan drug** by the NHS, **requires a standardised process**. This process requires transparency, consistency, and accountability to be perceived as legitimate by the public and health care providers to increase the likelihood of making right decisions.
- Decision-making related to orphan drugs (ODs) must face challenges regarding its evidence such as the lack of a treatment comparator, the evidence based on low number of patients or high prices. Thus, the evaluation of ODs needs highlight other criteria such as ethics, unmet needs, disease severity or patient's quality of life, among others.
- 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 and homogenise the assessment of ODs by the decision-making committee of the Catalan Health Service (CatSalut).

METHODS

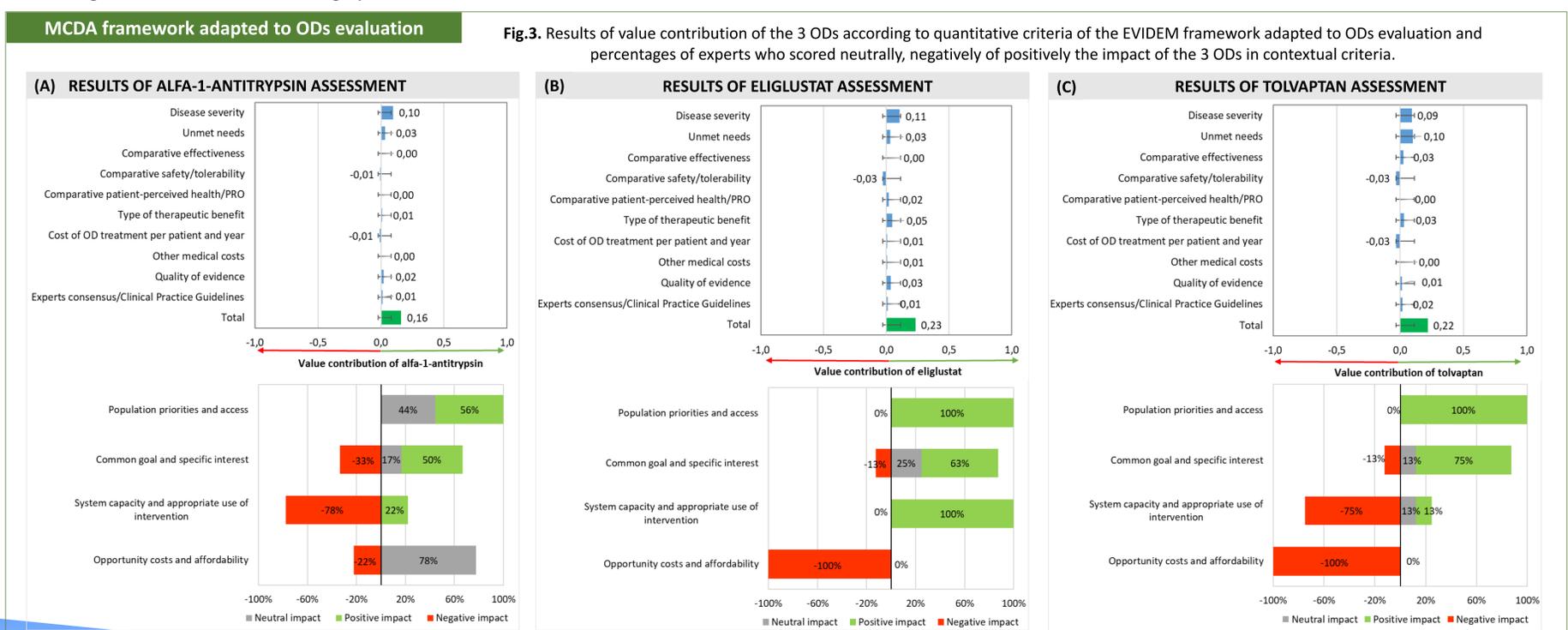
- A **preliminary framework based on EVIDEM (v.4.0)¹** and adapted according to a literature review² was **developed for its use in ODs evaluation** and was validated by representatives of the decision-making committee of CatSalut³.
- The **final version of the framework was agreed, weighted and tested by the committee members 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 through a direct rating scale (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 of the drug** by CatSalut.



CONCLUSIONS

A final MCDA framework specific for ODs was developed and validated to be used for the ODs evaluations conducted by the CatSalut. The test of the three OD through the developed framework specific for OD showed that MCDA can be considered a useful methodology which adds transparency, standardisation and allows a structured discussion that substantiates decision making adopted by the committee.

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