VALUE CONTRIBUTION OF DONIDALORSEN FOR THE LONG-TERM PROPHYLAXIS OF HEREDITARY ANGIOEDEMA IN SPAIN: A MULTI-CRITERIA DECISION ANALYSIS (MCDA)

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INTRODUCTION

Hereditary angioedema (HAE) is a rare, severe, and potentially life-threatening genetic disease characterized by recurrent and unpredictable attacks associated with pain, functional impairment, and substantial psychological burden[1-3].

The increasing availability of long-term prophylactic (LTP) options has made treatment selection more complex[4,5] .Donidalorsen is a new option for routine prevention of recurrent HAE attacks in adults and adolescents aged 12 years and above[6].

In this context, multicriteria decision analysis (MCDA) provides a structured and transparent framework to assess the value of treatment options across multiple dimensions relevant to decision-making[7-9].

OBJECTIVE

To assess the value contribution of donidalorsen for routine prevention of recurrent HAE attacks in adults and adolescents aged 12 years and above in Spain using an MCDA framework.

COMPARATORS

- pdC1INH intravenous (i.v.)
- pdC1INH Subcutaneous (s.c.)
- Berotralstat
- Lanadelumab
- Garadacimab

METHODS

Study design & framework

A structured MCDA was conducted following ISPOR good-practice recommendations[8,9] .

The assessment was based on the EVIDEM framework, which includes quantitative and contextual criteria[10,11].

Criteria selection & weighting

Multidisciplinary scientific committee (SC), including clinicians, hospital pharmacists, a nurse, a psychologist, and a healthcare decision maker, selected relevant EVIDEM criteria based on a targeted literature review.

Members allocated 100 points across quantitative criteria according to their relative importance.

Evidence matrix & scoring

The evidence was incorporated into a matrix, with each committee member independently assigning scores.

Non-comparative criteria used a 0–5 scale. Comparative criteria used −5 to +5, with positive scores favoring donidalorsen. Contextual criteria were classified as negative, neutral, or positive.

Due to the unavailability of cost data for some treatments, treatment cost was not included in the value contribution analysis.

Value contribution analysis

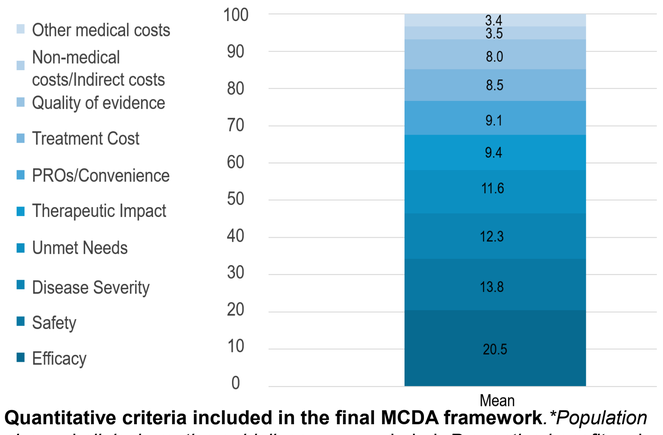
Responses were analyzed using descriptive statistics. Normalized weights and scores were combined to estimate each criterion's contribution and the overall value contribution, with higher values indicating greater overall value.

The primary analysis excluded intervention cost because acquisition costs were unavailable for some comparators. The committee summarized and interpreted results descriptively.

RESULTS

1. Criteria selection and weighting

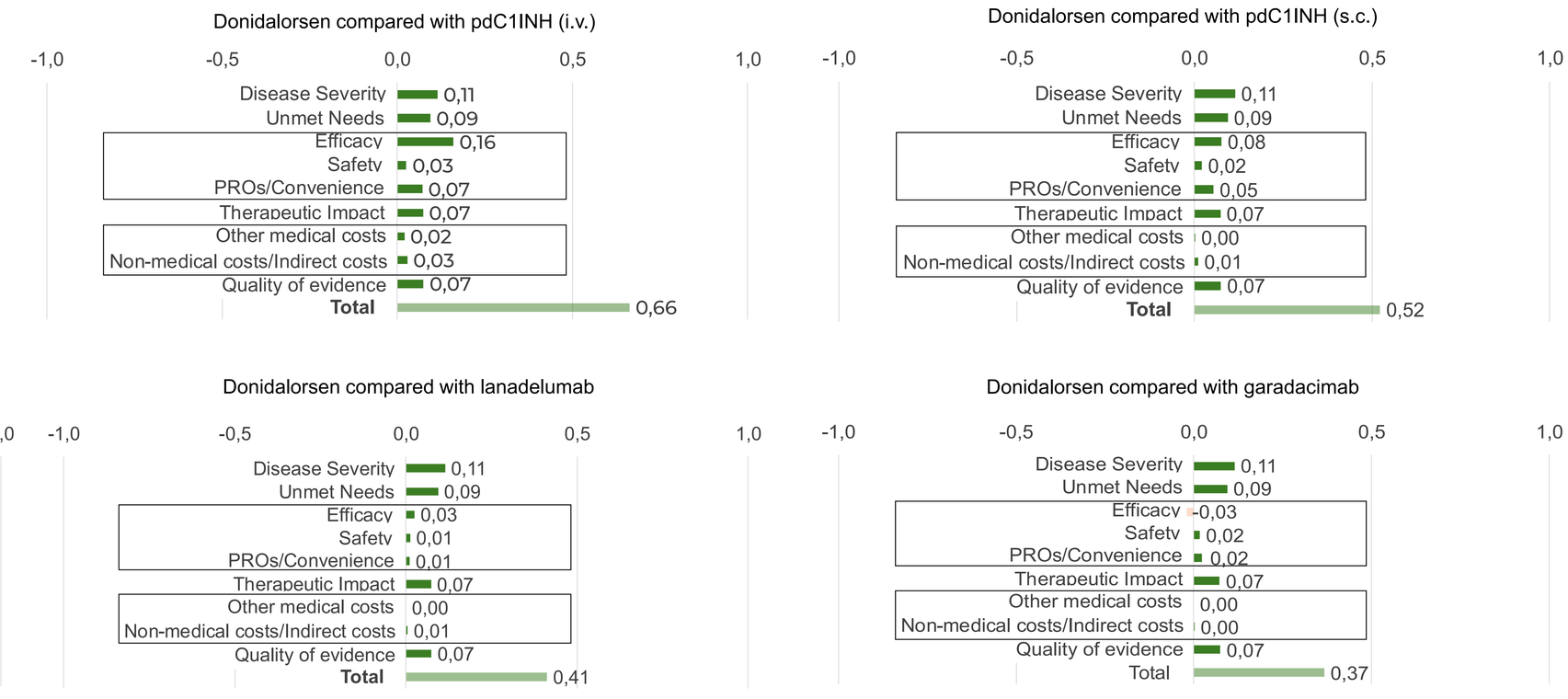
A total of 10/13 quantitative criteria were selected by the SC*. Efficacy and safety received the highest weights, while disease severity and unmet need were also considered relevant.



Quantitative criteria included in the final MCDA framework.**Population size* and *clinical practice guidelines* were excluded. *Preventive benefit* and *therapeutic benefit* were not retained as separate criteria but were merged into a single criterion, *therapeutic impact*. **PROs**, patient-reported outcomes;

2. Overall value contribution

- Donidalorsen showed a positive value contribution versus all comparators.
- Donidalorsen offered advantages in efficacy, safety, patient-reported outcomes, and indirect costs compared to pdC1INH i.v., pdC1INH s.c., and berotralstat and was broadly comparable to lanadelumab and garadacimab, providing added value in dosing flexibility.
- Dosing flexibility, including the potential to extend administration to every 2 months in well-controlled patients, was identified as an additional value driver



Value contribution of donidalorsen versus each comparator. Boxed criteria correspond to comparative criteria. **PROs**, patient-reported outcomes; **pdC1INH**, plasma-derived C1 inhibitor; **i.v.**, intravenous; **s.c.**, subcutaneous;

CONCLUSIONS

- Donidalorsen showed a positive value contribution for routine prevention of recurrent HAE attacks in Spain.
- Its clinical profile and flexible subcutaneous dosing, with monthly administration and the potential for every-2-month dosing in well-controlled patients, support its consideration as a valuable prophylactic option in clinical practice.

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