

# A Multi-Criteria Decision Analysis (MCDA) to determine the value of recombinant ADAMTS13 vs Standard of Care in Congenital Thrombotic Thrombocytopenic Purpura (cTTP) in Spain

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## INTRODUCTION

- MCDA is a **methodological tool** that explicitly considers **multiple factors relevant to decision-making**, being particularly useful for rare diseases.<sup>1,2</sup>
- cTTP is an **ultra-rare** prothrombotic disorder, characterized by a **severe congenital deficiency of ADAMTS13**, associated with **life-threatening acute episodes** and **long-term chronic sequelae**.<sup>3,4</sup>
- Current treatment for cTTP primarily consists of **plasma infusions**, either on-demand or as regular prophylaxis, which are **ineffective at fully replenishing normal ADAMTS13 levels**.<sup>5</sup>
- Recombinant ADAMTS13 (rADAMTS13) is a **newly approved orphan treatment for cTTP** and the only specifically developed for this disease.<sup>6</sup>

## OBJECTIVE

To assess the **value contribution of rADAMTS13 versus** current standard of care in the **treatment of cTTP in Spain using a MCDA methodology**.



## METHODS



A **literature review** was performed to populate an **evidence matrix**, composed of quantitative and contextual criteria, included in a MCDA framework specifically adapted to appraise Orphan Drugs in Spain (**EVIDEM framework**).<sup>7</sup>



A **multidisciplinary expert panel of nine experts** (haematology, hospital pharmacy, nursing, hospital management, health economics, patient advocacy, and psychology) **scored the criteria** of the MCDA framework.

- According to the type of criteria, different qualitative and quantitative scoring scales were used, with higher scores representing higher value.



Results were analysed and discussed in a **group meeting**.

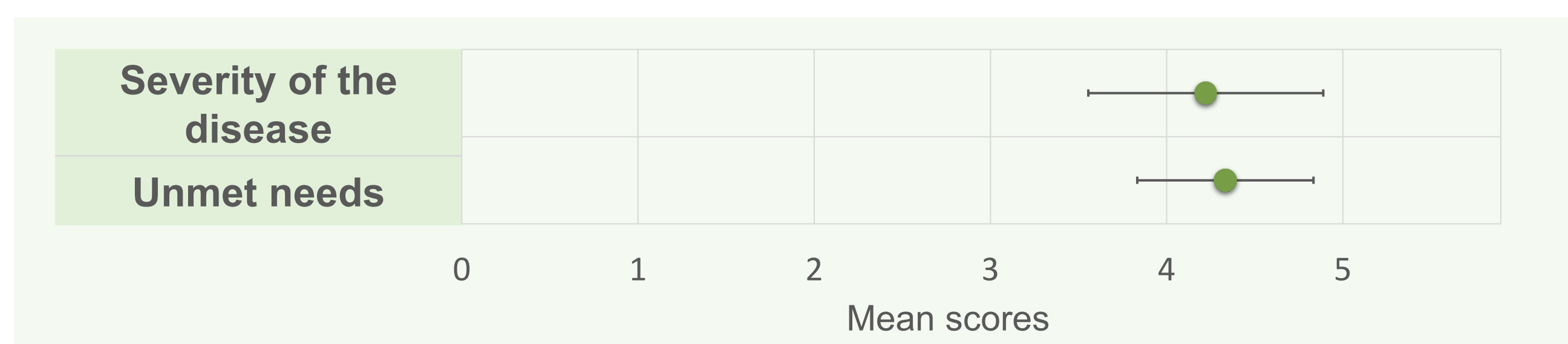


Scores were weighted to assess the **overall value contribution of rADAMTS13** against standard of care (plasma-based therapies).

## RESULTS

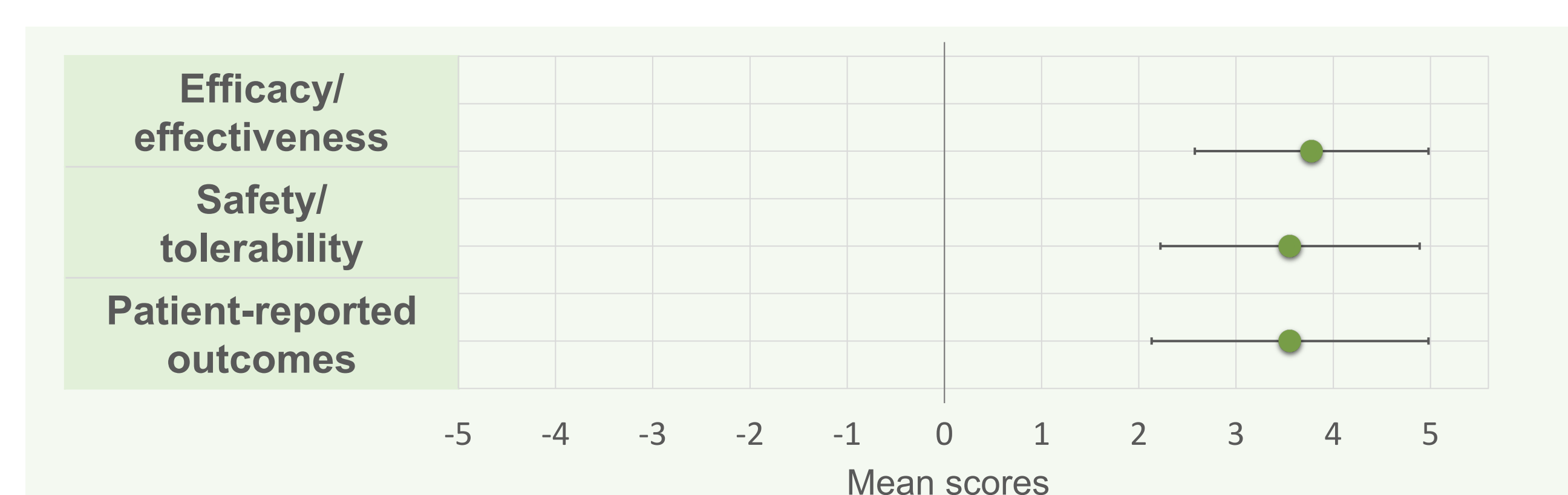
### Disease related criteria

- Disease related criteria were **rated high for rADAMTS13 in both unmet needs** (mean: 4.3/5 ± 0.5) and **severity of the disease** (4.2/5 ± 0.7).

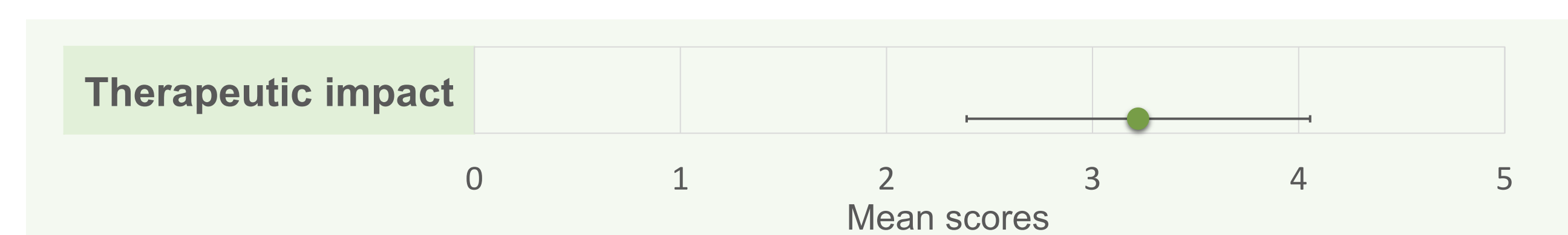


### Treatment related criteria

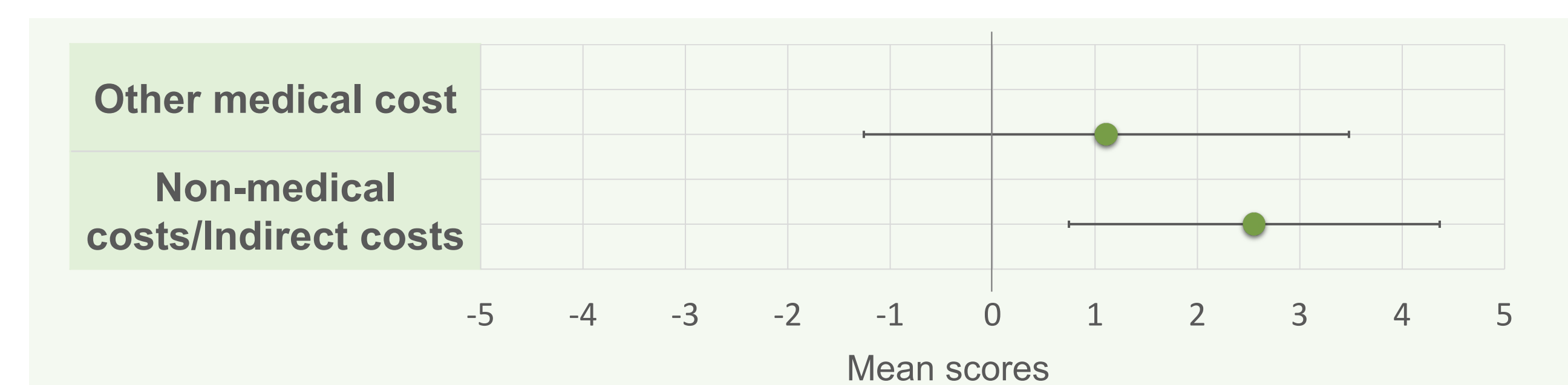
- rADAMTS13 was **perceived as a more effective** therapeutic option, with a **superior safety profile and tolerability**, and the **ability to provide significant improvement in quality of life** compared with standard of care (3.8 ± 1.2, 3.6 ± 1.3, and 3.6 ± 1.4, respectively, on a scale of -5 to 5).



- Most experts agreed on the **positive therapeutic impact of rADAMTS13** (3.2 ± 0.8, range 2–4; scoring scale 0 to 5).

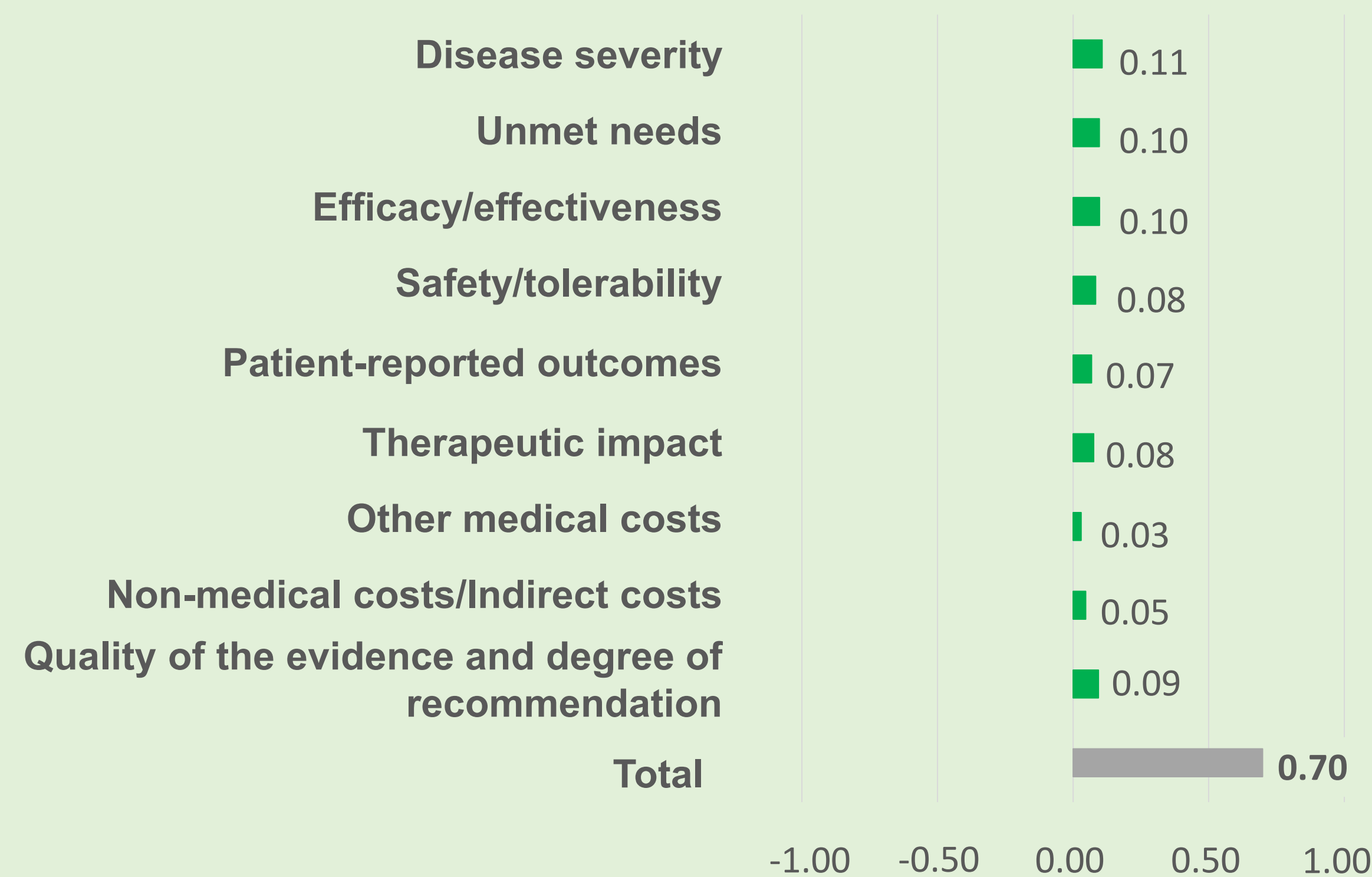


- rADAMTS13 could result in **lower other medical costs and indirect costs** (1.6 ± 1.8 and 2.8 ± 1.7, respectively), mainly based on the **shorter time required to administer rADAMTS13**.



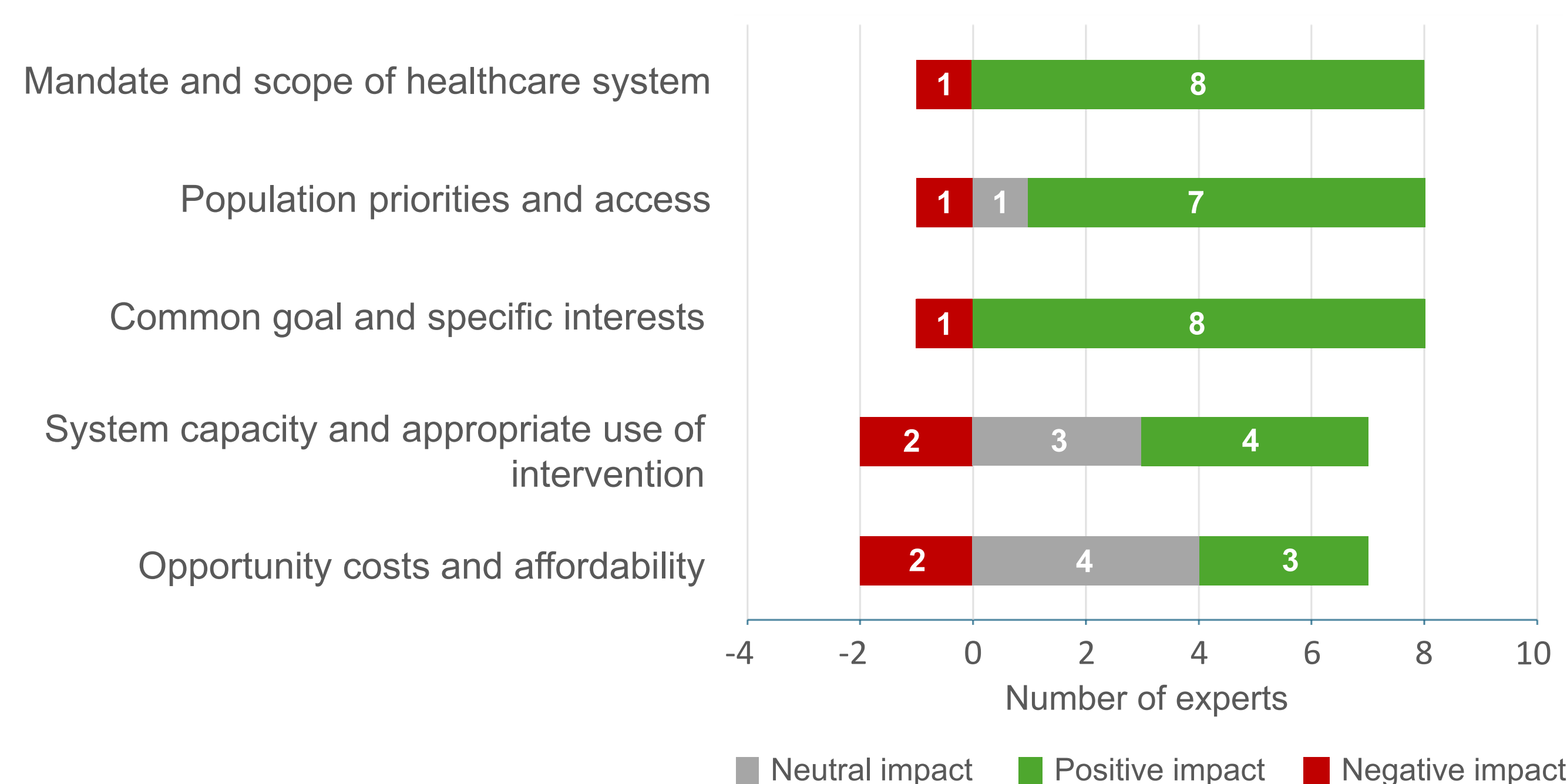
### Overall evaluation of the contribution of rADAMTS13

- The weighted **value contribution of rADAMTS13 to the management of cTTP was scored as 0.7** on a scale from -1 to 1.



### Contextual criteria

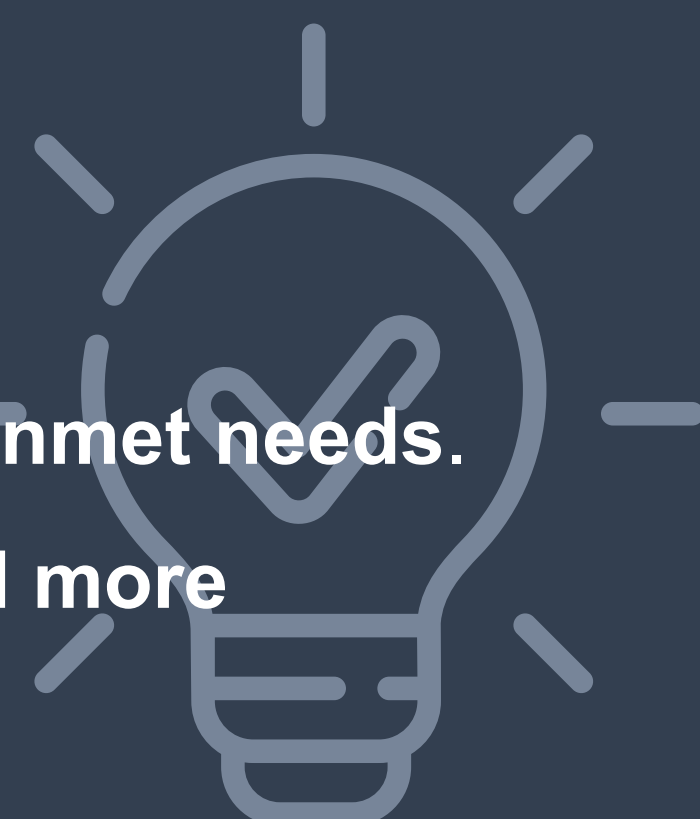
- The **overall qualitative criteria showed a positive impact**, except for the neutral impact of opportunity and affordability costs criterion.



With an overall value contribution of 0.7, rADAMTS13 positions very positively within the value range assigned to orphan drugs in an analysis conducted in Spain by the Working Group on Rare Diseases and Orphan Drugs (Orphar-SEFH Group), where values ranged from 0.2 to 0.8 on a scale from -1 to +1.<sup>8</sup>

## CONCLUSIONS

- cTTP is recognized as a **severe disease with important unmet needs**.
- rADAMTS13 is **perceived as a more effective, safer and more convenient therapeutic option than standard of care**.



## REFERENCES

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