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Value drivers for therapies in Fabry disease: findings from a European payer and clinician study

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Introduction

- Fabry disease is a rare, progressive and multisystemic lysosomal disorder.[1]
- Despite available treatments, there remains a substantial burden in terms of quality of life (QoL) for people living with Fabry disease and their carers. For payers, healthcare resource use and treatment costs are major issues.[1,2]
- The disease management approach has recently shifted towards earlier diagnosis and intervention, with the aim of preserving organ function and improving QoL and survival.[1,3]
- This research examined European payer and clinician perspectives of the unmet treatment need, the burden of illness associated with Fabry disease, and healthcare cost drivers in order to understand and prioritise value drivers for treatment.

Conclusions

- There remain important unmet needs in the treatment of Fabry disease from both the payer and clinician perspectives. These relate to reducing morbidity and improving control of symptoms and clinical events, including reduction in renal, cerebrovascular and cardiac events.
- There are opportunities for delivering new value in Fabry therapies through efficacy improvement. Payers and clinicians considered the most influential value drivers to be multisystemic (overall) efficacy, cardiac and renal efficacy, improvement in QoL, and reduction in pain symptoms. Payers also valued cost-effectiveness and affordability more highly than clinicians.
- In some countries, payers and clinicians attributed greater value to mode of action and route of administration than in other countries
  - In some countries, regional payers attributed greater value than national payers to storage requirements, as well as special requirements (eg fasting, pre-medication).
- As the first comprehensive study with European payer experts and clinicians on value drivers in Fabry disease, this research revealed opportunities for further improvement and innovation to address the remaining unmet needs.

Methods

- A double-blind interview programme was conducted in eight countries (France, Germany, Italy, Poland, Portugal, Spain, Sweden, and the UK) between October and December 2023, spanning both quantitative and qualitative interviews. Eligible stakeholders were selected using a screener
  - 1:1 qualitative telephone interviews with 28 stakeholders (four per country comprising three payers and one clinician). Stakeholders were asked to:
    - Provide qualitative feedback about the burden of Fabry disease and the unmet treatment needs.
  - 1:1 quantitative structured surveys with 120 stakeholders (15 per country comprising 10 payers and five clinicians). Stakeholders were asked to:
    - Characterise the burden of disease from minor to critical in terms of severity (minor, moderate, serious, severe, critical)
    - Score a list of unmet treatment needs from 1 (low unmet need) to 5 (high unmet need).
- For both surveys and interviews, stakeholders were asked to rate a list of value drivers when evaluating a medicine for market access and pricing decision-making on a scale from 1 (not important) to 5 (highly important).
- Please refer to the Supplement, accessible via the QR code, for further information on the methodology.

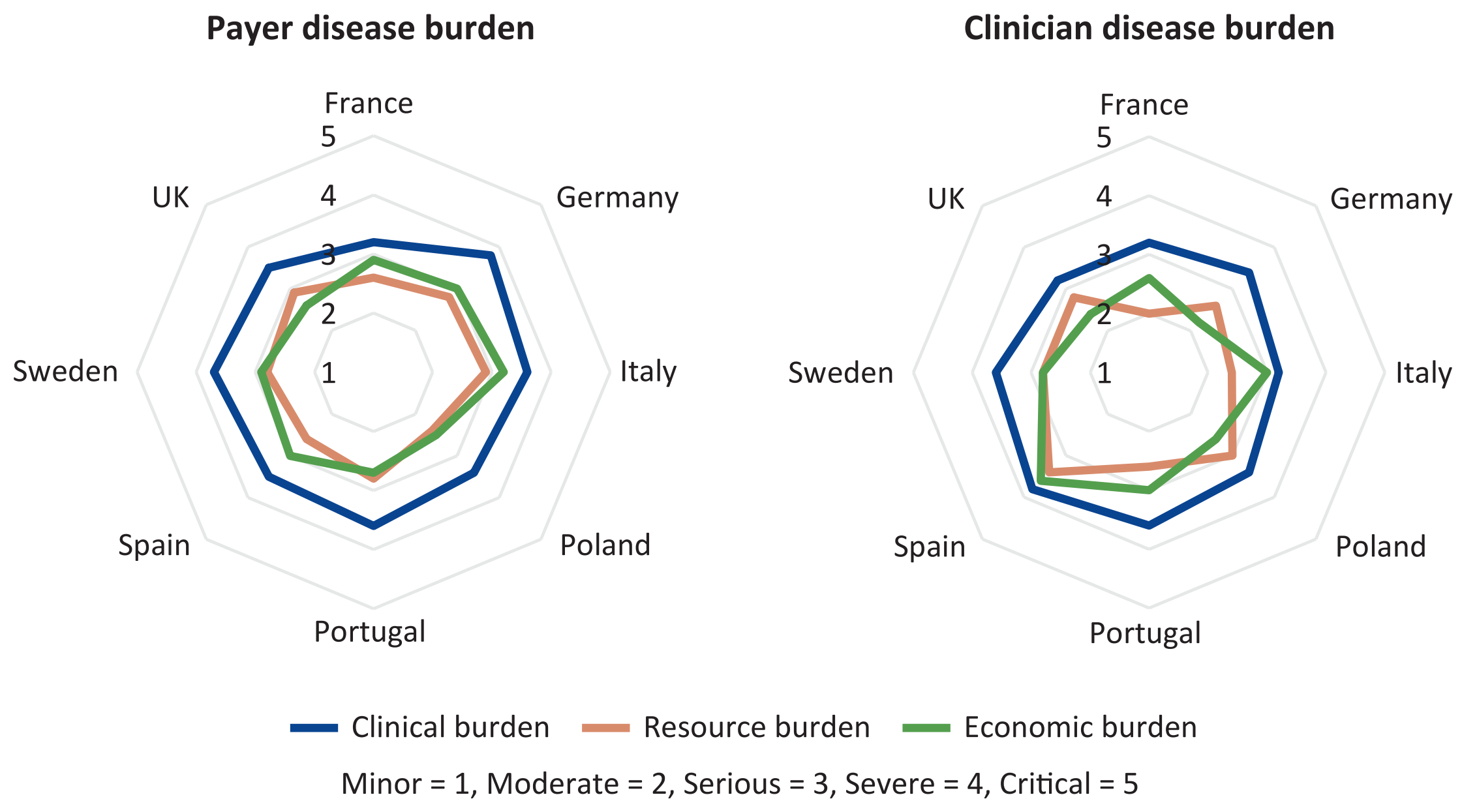
Results

Demographics

- The majority of payers or payer experts who participated in the research were national (67%) rather than regional (33%). Both current payers and payer experts (former payers) were included in accordance with local compliance and institutional rules in the relevant country.
- As Fabry is a multisystemic disease, multiple clinician specialties were represented. Clinician specialities included neurology (39%), rare disease (20%), cardiology (20%), inherited metabolic disease (4%), nephrology (4%), and other (13%).

Disease burden

*Payers and clinicians perceived the clinical burden of Fabry disease to be serious-to-severe, and the resource and economic burdens to be moderate-to-severe**

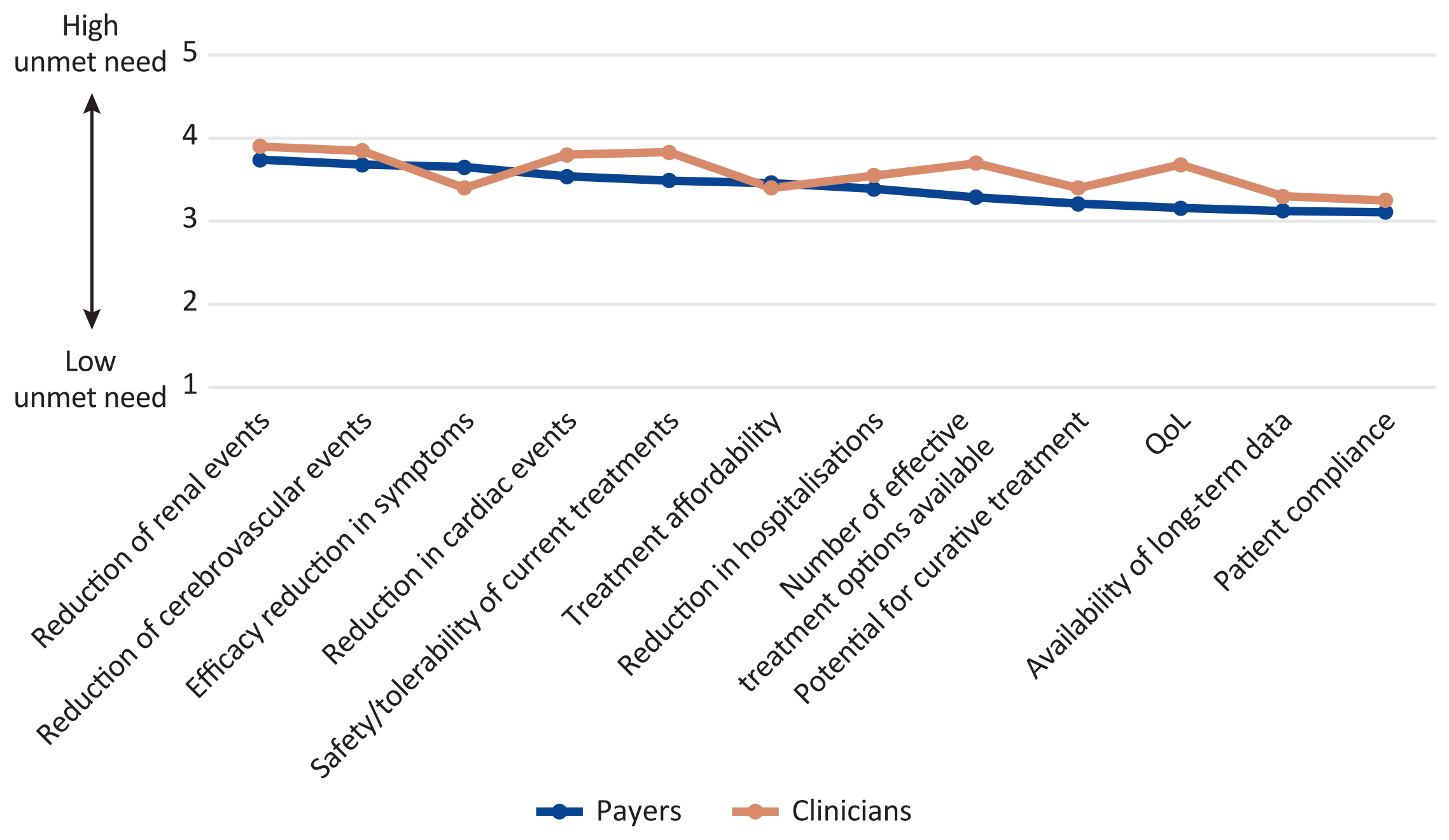


*Survey stakeholders were asked to score disease burden as follows: Minor = 1, Moderate = 2, Serious = 3, Severe = 4, Critical = 5.

- Fabry disease heterogeneity was emphasised, with some respondents classifying clinical burden as extremely severe for patients with cardiac or renal issues.
- Respondents mentioned that patients have reduced life expectancy and experience loss of QoL, particularly for males.
- The resource burden was viewed as higher for patients with more severe disease.
- Payers highlighted the high costs of treating complications such as cardiac or renal issues, but noted that the overall budget impact was manageable because of the rarity of the disease.

Unmet needs

*Reductions in renal, cerebrovascular and cardiac events were rated as key unmet needs by both payers and clinicians**

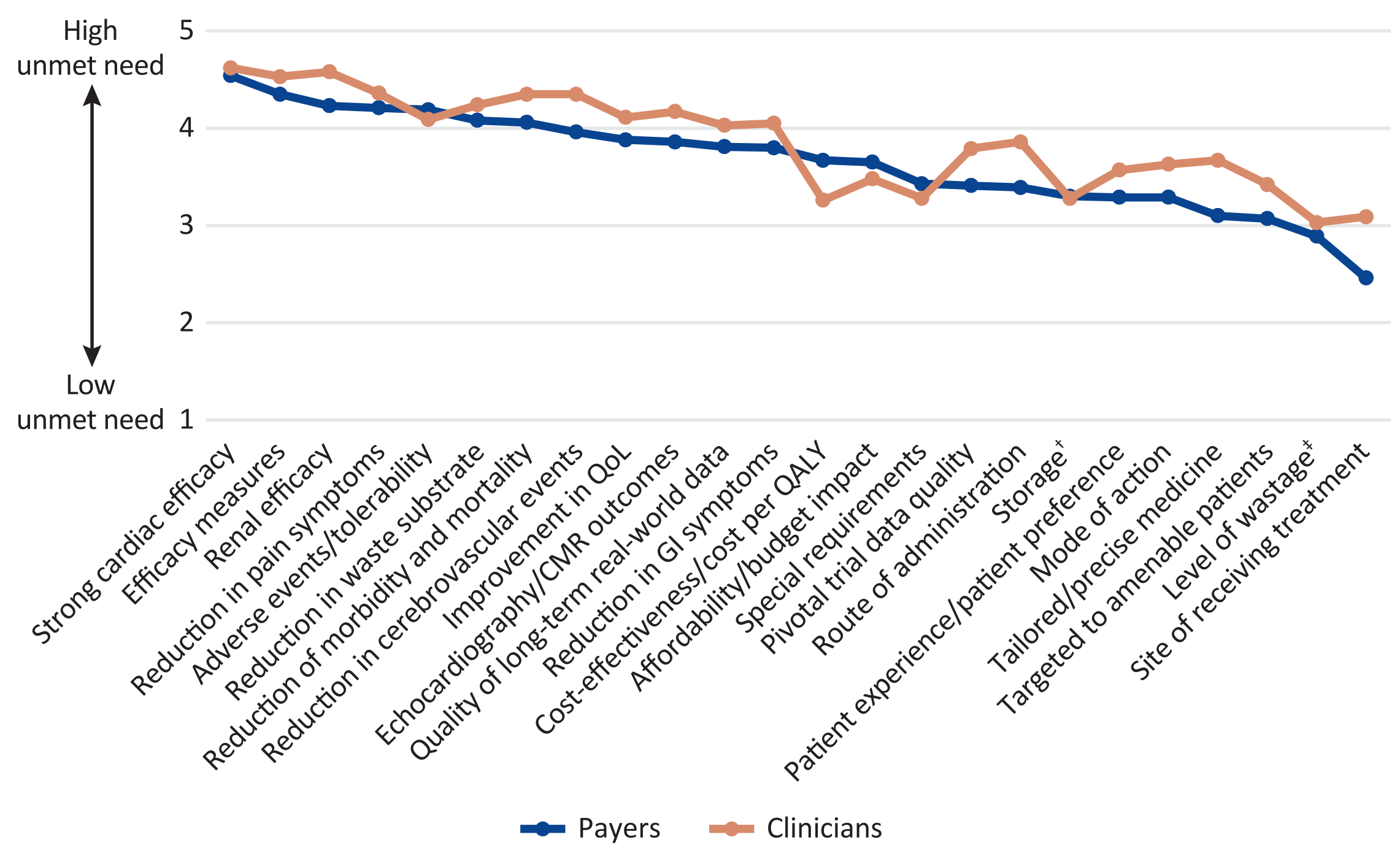


*Survey stakeholders were asked to score a list of unmet needs on a scale from 1 (low unmet need) to 5 (high unmet need).

- Payers perceive the key unmet needs to be the reduction in renal, cerebrovascular and cardiac events, as well as reduction of overall symptoms.
- Clinicians prioritised similar unmet needs as payers, as well as the safety and tolerability of current treatments.

Value drivers

*Both payers and clinicians rated most value drivers with a score of at least 3 out of 5, indicating moderate-to-high importance**

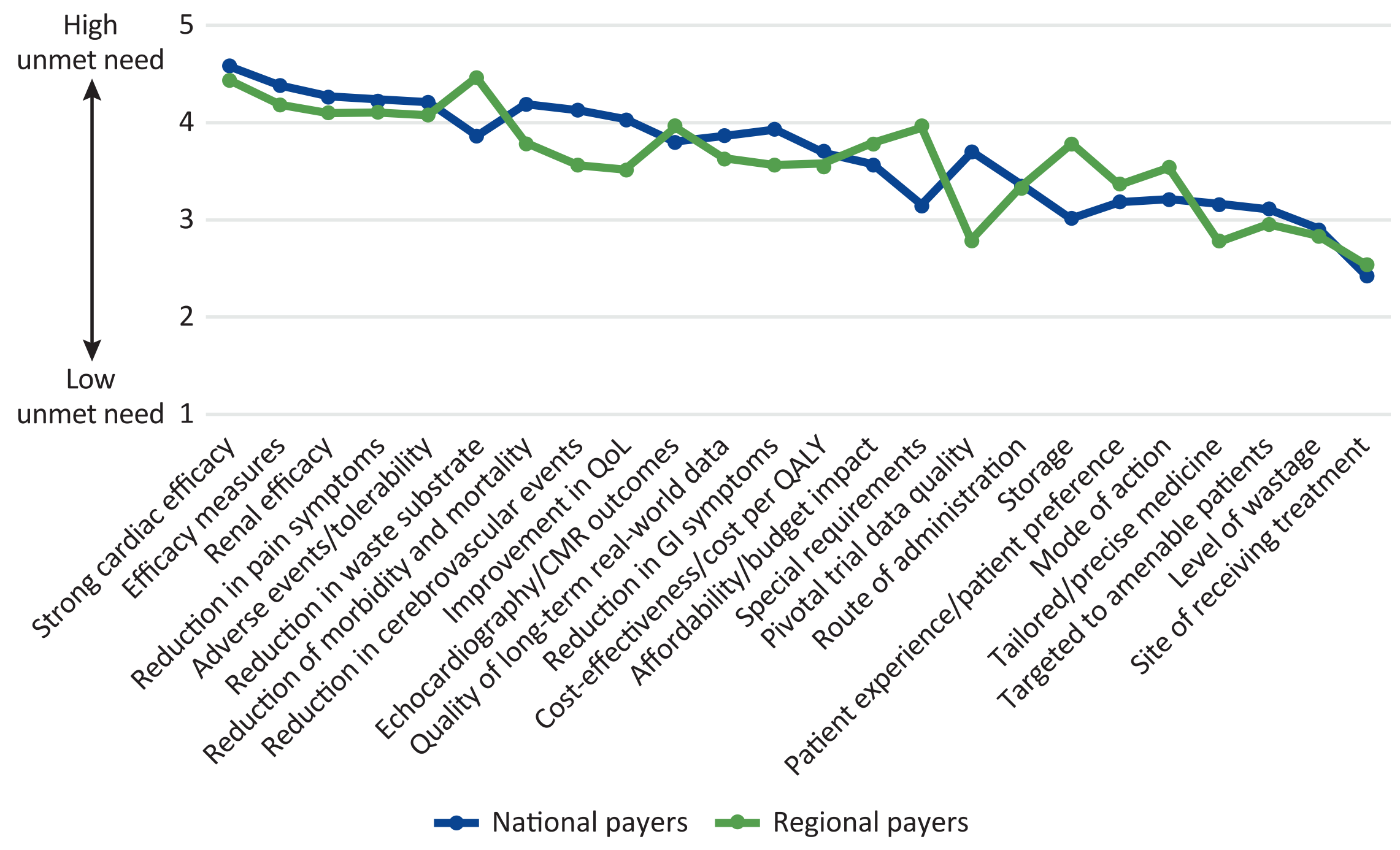


*For the survey and interviews, stakeholders were asked to rate a list of value drivers on a scale from 1 (not important) to 5 (highly important) in the context of evaluating a drug for market access and pricing decision-making; †Refers to storage of the drug; ‡Refers to product wastage when reconstituting for administration.

CMR, cardiac magnetic resonance; GI, gastrointestinal; QALY, quality-adjusted life year.

- Both payers and clinicians ranked multisystemic (overall) efficacy, cardiac and renal efficacy, and reduction in pain symptoms as the most influential drivers across all countries.
- Payers and clinicians were also aligned on the value drivers they considered to be the least influential, with the site of receiving treatment and level of wastage (product wastage when reconstituting for administration) the lowest rated.
- Payers rated all value drivers slightly lower than clinicians, except for storage (storage of drug), adverse events and tolerability, special requirements (eg fasting, pre-medication), affordability/budget impact, and cost-effectiveness/cost per QALY.

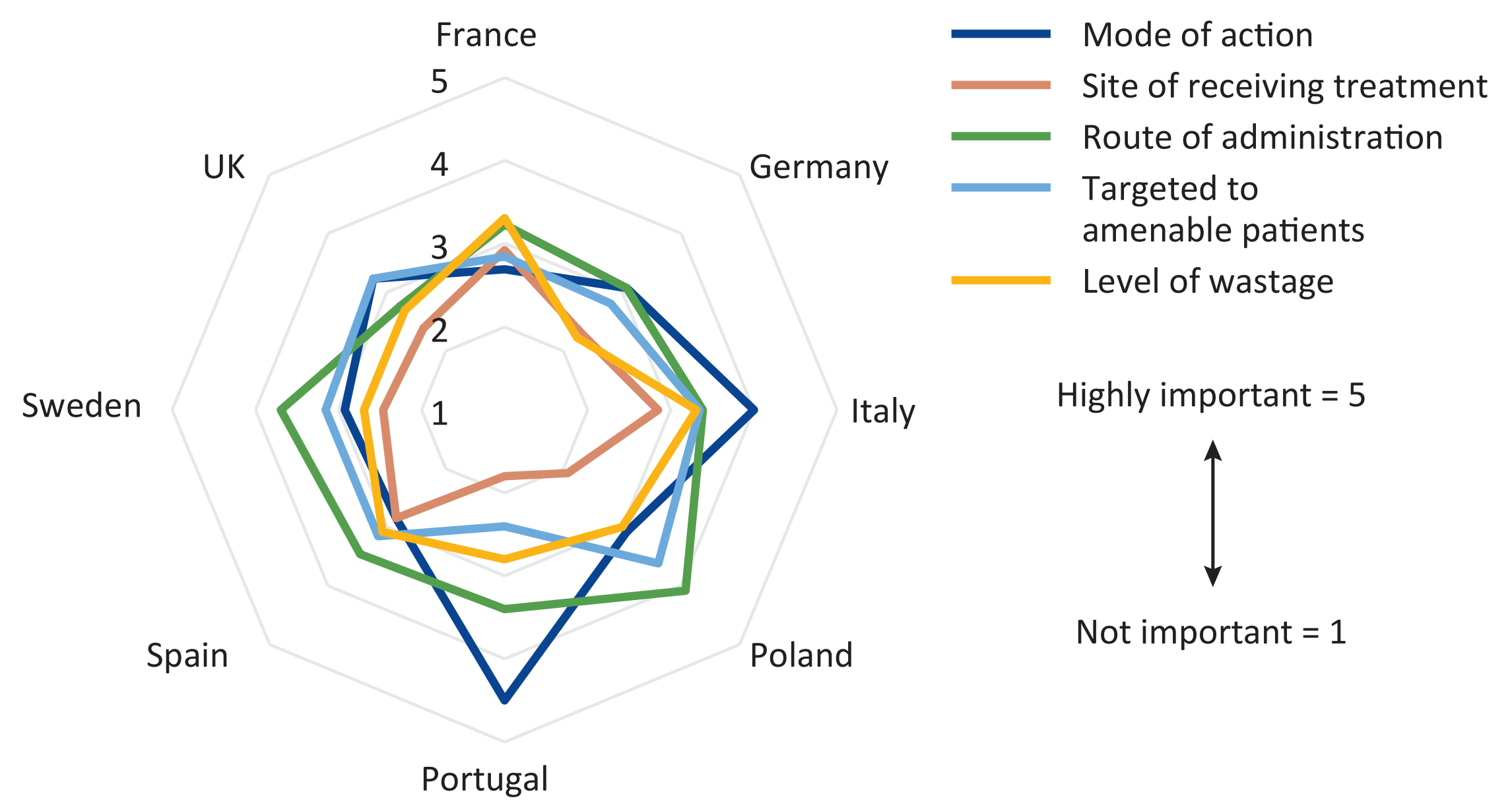
The importance of some value drivers varied considerably for national and regional payers



*For the surveys and interviews, stakeholders were asked to rate a list of value drivers on a scale from 1 (not important) to 5 (highly important) in the context of evaluating a drug for market access and pricing decision-making.

- Regional payers attributed greater value than national payers to storage requirements, as well as special requirements (eg fasting, pre-medication).
- On the other hand, regional payers attributed less value than national payers to efficacy measures and pivotal data quality.

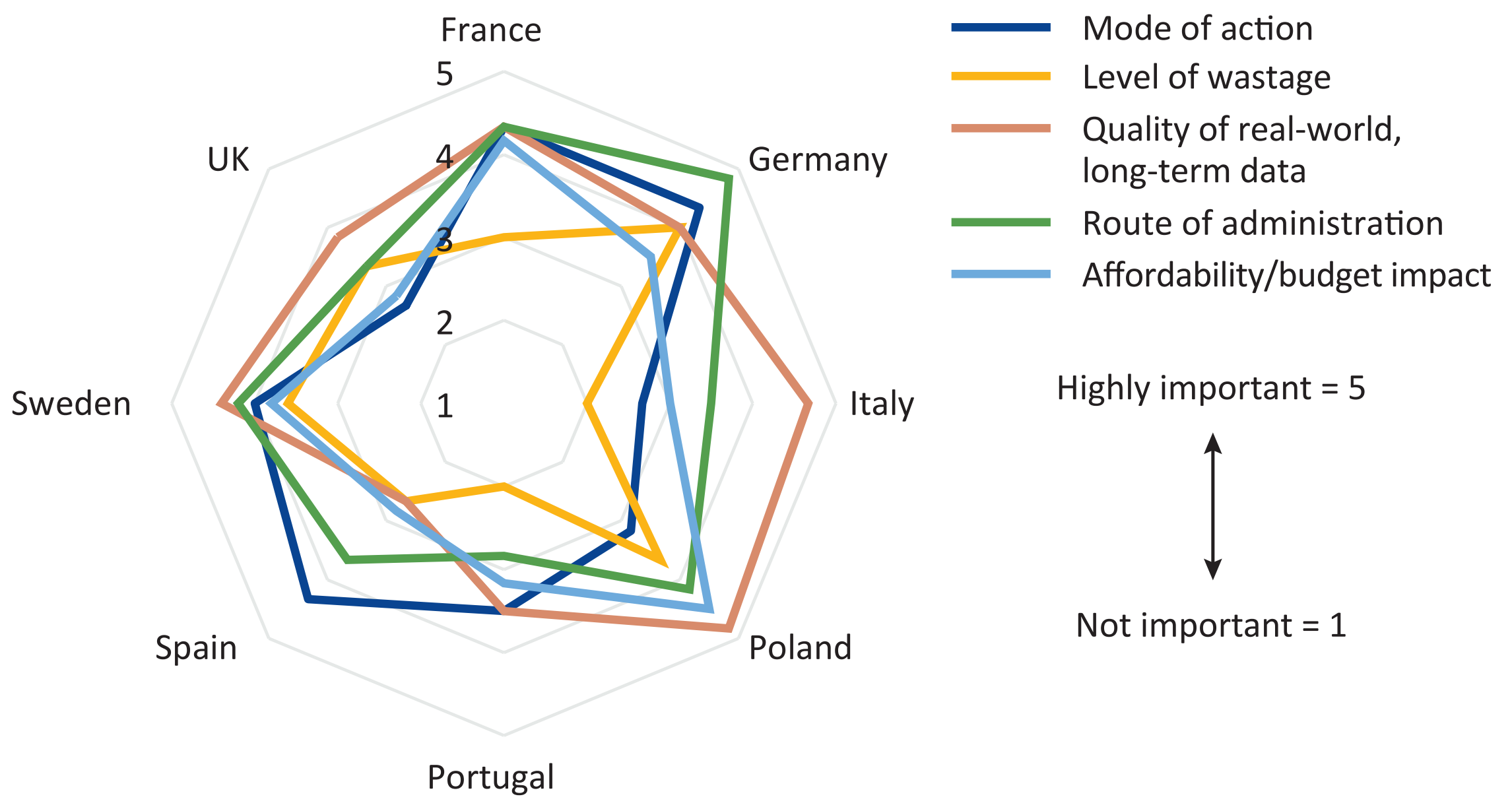
Payers in some countries rated the mode of action and route of administration as more important than in other countries,†*



*For the surveys and interviews, stakeholders were asked to rate a list of value drivers on a scale from 1 (not important) to 5 (highly important) in the context of evaluating a drug for market access and pricing decision-making; †This figure represents the five drivers that showed the most variation in payer response across countries.

- Payers in Portugal and Italy attributed greater value to mode of action than in other countries.
- Route of administration was rated as more important in Poland and Sweden than it was in other countries.

Clinicians showed greater intercountry variation than payers, with clinicians in some countries rating mode of action as more important than in other countries,†,‡*



*For the surveys and interviews, stakeholders were asked to rate a list of value drivers on a scale from 1 (not important) to 5 (highly important) in the context of evaluating a drug for market access and pricing decision-making; †This figure represents the five drivers that showed the most variation in clinician response across countries; ‡Level of wastage refers to product wastage when reconstituting for administration.

- Mode of action was more highly rated as a value driver among clinicians in France, Germany, Spain, and Sweden than in other countries.
- High variation was observed in rating the level of wastage (product wastage when reconstituting for administration), with Germany ranking it especially highly.
- Quality of real-world, long-term data was generally considered an important value driver across countries, apart from in Spain.

References

1. Jovanovic A *et al. Orphanet J Rare Dis* 2024;19:181.
2. Gold KF *et al. Qual Life Res* 2002;11:317–27.
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