Reasons for a Negative Pharmaceutical Benefits Advisory Committee (PBAC) Review in Rare Diseases in Australia: A Comprehensive Analysis

HTA23

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SUMMARY

OBJECTIVES

- The Pharmaceutical Benefits Advisory Committee (PBAC) plays a crucial role in evaluating the clinical and cost-effectiveness of medications for reimbursement in Australia to try and ensure efficient allocation of healthcare resources.
- The process for rare diseases can pose unique challenges due to evidence limitations and high costs.
- The objective of this study was to identify and analyse the reasons for negative PBAC reviews in rare diseases, focusing on the objectives, methods, results, and conclusions of the assessment process.

METHODS

- This study involved a comprehensive review and analysis of publicly available PBAC meeting outcomes, guidelines, and relevant literature on orphan drug evaluations published between 2021 and 2023.
- The identified evaluations were examined to highlight common reasons for negative reimbursement decisions by PBAC.

FINDINGS

- The review identified 22 negative PBAC recommendations across 20 different orphan medications by PBAC between 2021 and 2023.
- The most frequent factors contributing to a negative outcome were high treatment costs (77%), uncertainties in estimating long-term clinical effectiveness (50%), limited clinical evidence (36%), and issues with economic modelling (32%)..

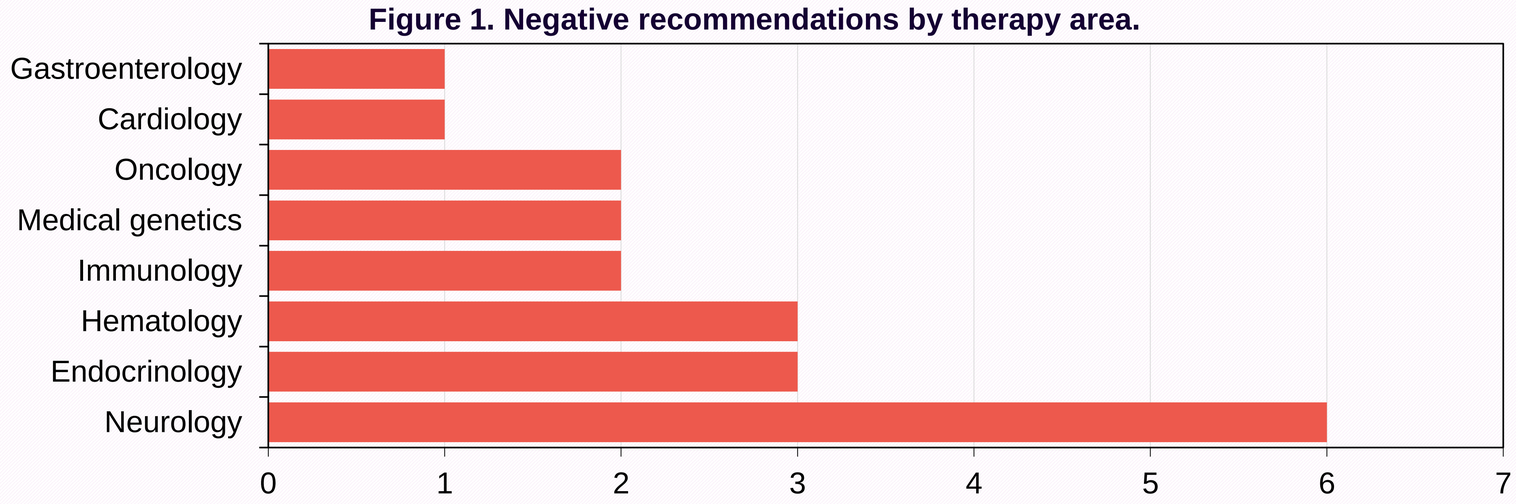
BACKGROUND & AIMS

- PBAC recommend new medications for reimbursement in Australia based on disease area, clinical effectiveness, safety, and cost-effectiveness compared with other treatments.[1]
- As part of Australia's Orphan drug program, the sponsor is eligible for a waiver of the fee for the first HTA submission to PBAC.[2]
- Due to the nature of rare diseases, issues arise during the HTA process due to evidence limitation, caused by small populations and lack of comparators, and high costs.
- This study examined orphan medications with which received negative PBAC recommendations and identified the reason for the outcome.
- **The objective of this study was to synthesise frequent reasons that PBAC make negative recommendations for orphan products, to help proactively mitigate these challenges and improve access to orphan medications in Australia.**

METHODS

- Publicly available PBAC meeting outcomes, guidelines, and relevant literature between 2021 and 2023 were screened to identify reimbursement decisions for orphan medications.
- Negative recommendations were identified, and data describing the treatment evaluated, disease area, and reasons for rejection were summarised.
- The identified evaluations were then qualitatively examined to explore common reasons for negative reimbursement decisions and identify the limitations that frequently result in negative recommendations from PBAC.

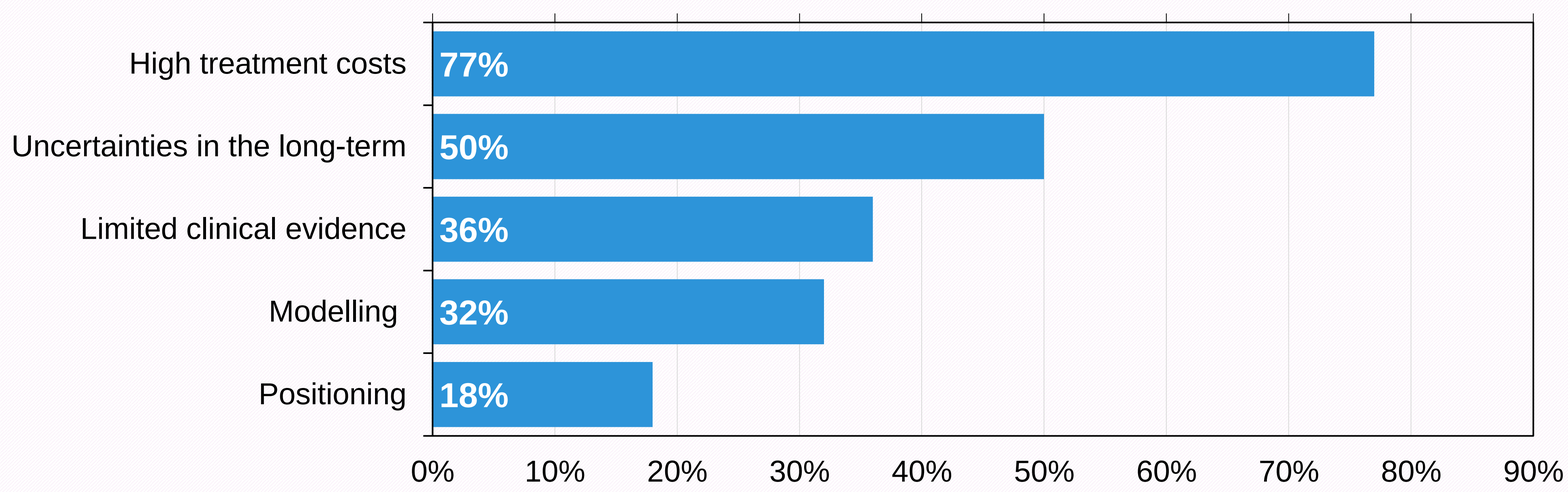
Figure 1. Negative recommendations by therapy area.



RESULTS

- This review identified 22 negative PBAC recommendations between 2021 and 2023.
- In total, 20 products were considered across 19 different indications, and 8 different disease areas: neurology (n = 6), haematology (n = 3), endocrinology (n = 3), immunology (n = 2), oncology (n = 2), genetic diseases (n = 2), gastroenterology (n = 1), and cardiology (n = 1). (**Figure 1**)
- The most frequent factors contributing to a negative outcome were high treatment costs, mentioned in 17 of the 22 recommendations (77%). (**Figure 2**)
- The other most frequent issues raised by PBAC were uncertainties in estimating long-term clinical effectiveness (n = 11, 50%), limited clinical evidence (n = 8, 36%), issues with economic modelling (7 = 32%), and the proposed positioning of treatments (n = 4, 18%).

Figure 2. Contributing factors to negative recommendations by PBAC.



- Additionally, variations in disease prevalence and heterogeneity, as well as limited patient populations and associated difficulties in conducting clinical trials contributed to negative recommendations from PBAC.

CONCLUSIONS

- This study highlights the primary reasons for negative PBAC reviews for orphan medications.
- Identifying frequent issues raised by PBAC highlights the challenges faced in assessing the clinical and cost-effectiveness of treatments for rare diseases.
- It is essential to explore alternative evaluation methodologies that can help mitigate the unique challenges of obtaining reimbursement for treatments for rare diseases in Australia, such as adaptive pathways, real-world evidence, and patient-reported outcomes.
- Collaborative efforts among stakeholders, including pharmaceutical companies, patient advocacy groups, and regulatory bodies, are necessary to overcome these challenges and ensure timely access to effective treatments for patients with rare diseases.

References

1. *PBS. (2023). PBAC Membership. https://www.pbs.gov.au/pbs/industry/listing/participants/pbac*
2. *TGA. (2018). Orphan drug designation. https://www.tga.gov.au/resources/resource/guidance/orphan-drug-designation*

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