

Patient Involvement in Rare Disease Health Technology Assessment (HTA): Real-World Practices in Australia, Singapore, and Taiwan

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Introduction

Patient involvement in health technology assessment (HTA) is crucial to ensuring that healthcare funding decisions are patient-centered, reflect real-world lived experiences and account for the values and preferences of the individuals most affected by those decisions.¹ It is especially critical in rare diseases, where evidence is thin and often limited by factors such as small patient populations and heterogeneous disease manifestations.^{2,3}

In Asia-Pacific (APAC), patient involvement in HTA is expanding. However, the extent and mechanisms of engagement vary across the region.³ Australia, Singapore, and Taiwan may be considered more advanced, having moved beyond ad hoc consultation toward more formalized mechanisms for patient participation in HTA. Yet even in these jurisdictions, the extent and impact of patient input on rare disease funding decisions remain difficult to discern.

Objectives

To assess the extent of patient involvement in rare disease HTAs and its alignment with formal patient involvement guidance across Australia, Singapore, and Taiwan.

Methods

We analyzed all rare disease HTA reports (not limited to new drug listings) published in 2025 by Australia's Pharmaceutical Benefits Advisory Committee (PBAC), Singapore's Agency for Care Effectiveness (ACE), and Taiwan's Center for Drug Evaluation (CDE). Rare disease status was classified using the Genetic and Rare Diseases Information Center (GARD) list.⁴

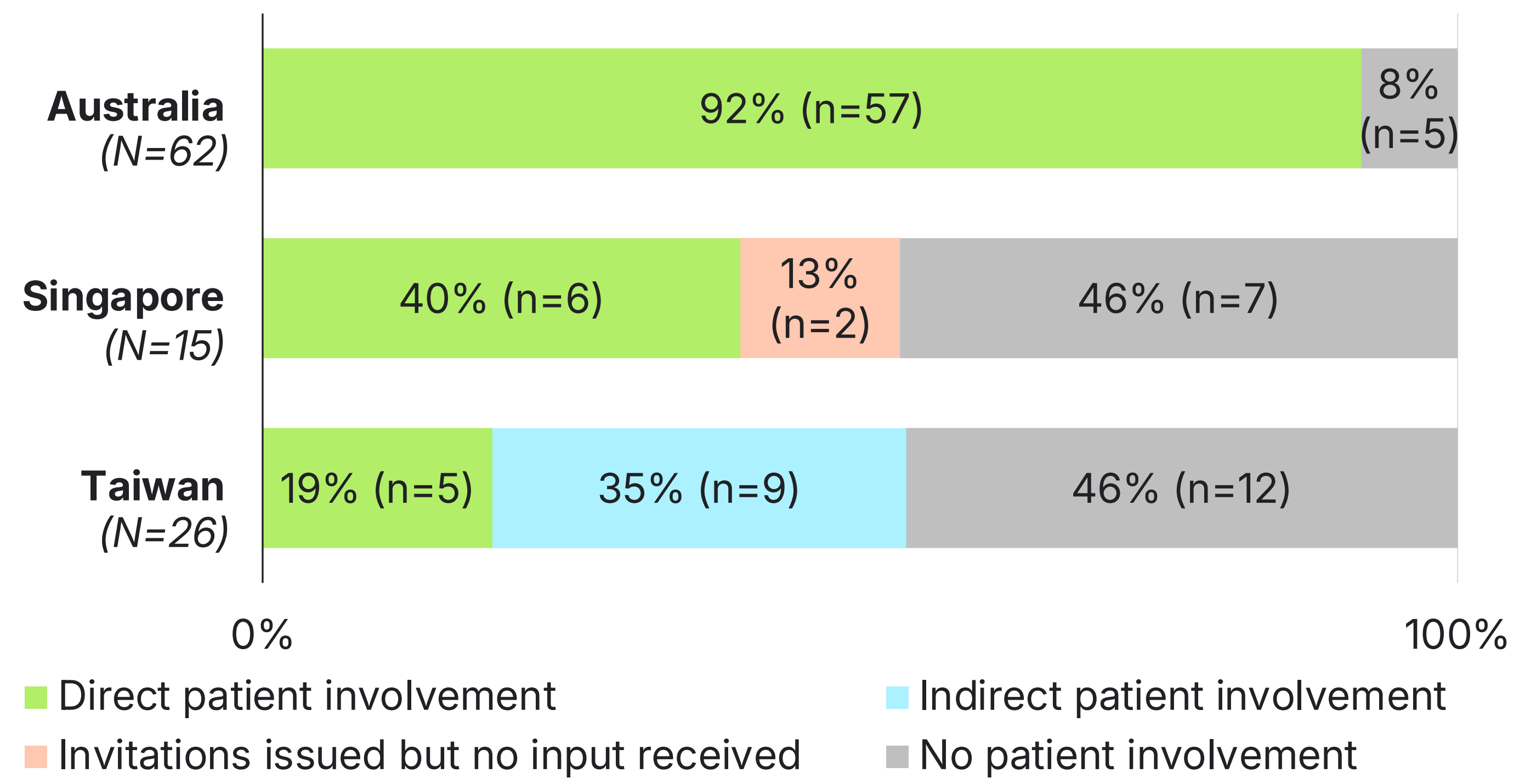
Across reports, we extracted structured data on whether and how patients were involved in HTA, the types of input collected, and the impact of patient input on reimbursement outcomes. Raw data were analyzed to identify within- and cross-jurisdictional trends. Real-world practice of patient involvement was evaluated against each jurisdiction's patient involvement guidance.

Results

Frequency of patient involvement in rare disease HTA

In 2025, PBAC, ACE and CDE published 62, 15 and 26 rare disease HTA reports, respectively. The extent of patient input varied markedly across jurisdictions (Figure 1). Patient involvement was most common in Australia, appearing in 92% of reports. In Singapore, 40% included patient testimonials, while two additional reports received no responses despite invitations. In Taiwan, 19% included direct local patient input, while 35% instead drew on patient evidence from foreign HTA agencies due to a lack of systematically collected local information.

Figure 1: Proportion of rare disease HTA reports published in 2025 that incorporated patient perspectives



Stakeholders and mechanisms of patient involvement

- Australia** received input from the broadest range of stakeholders. Patients, caregivers, patient organizations, and members of the public provided written input through the Pharmaceutical Benefits Scheme (PBS) Consumer Comments facility (the dominant channel) or the newer Office of Health Technology (OHTA) Consultation Hub.
- Singapore** invited patient experts from registered patient organizations or voluntary welfare organizations to submit testimonials.
- Taiwan** collected structured input from patients, caregivers and patient organizations through the National Health Insurance (NHI) Patient Opinion Sharing Platform. Patient organizations often conducted their own surveys or questionnaires and submitted aggregate results through the platform.

Type of evidence collected for rare disease HTA

Across the jurisdictions' 68 reports with direct patient involvement, patient evidence was predominantly descriptive. Treatment experience (90%), quality of life/daily functioning (81%), and disease/symptom burden (72%) were the most frequently reported types of patient input (Table 1). While Australia captured the widest range of patient input types, this may reflect its larger number of reports, rather than a narrower scope of patient input in Singapore or Taiwan.

Table 1: Types of patient input reported in assessments with direct patient involvement

Type of patient input	Australia (N=57)	Singapore (N=6)	Taiwan (N=5)	Total (N=68)
Treatment experience	91%	100%	60%	90%
Quality of life/daily functioning	77%	100%	100%	81%
Disease/symptom burden	72%	50%	100%	72%
Psychological/emotional impact	44%	83%	60%	49%
Treatment preferences	46%	0%	40%	41%
Unmet need/few treatment options	47%	0%	20%	41%
Carer burden	26%	0%	60%	26%
Pediatric/family concerns	19%	0%	0%	16%
Policy/access restrictions	14%	0%	0%	12%

Legend: ≥70% 30-69% <30%

Impact of patient involvement on rare disease funding

- Across all three jurisdictions, patient input was generally used to characterize disease burden and unmet need, with no explicit statement of its impact on the HTA recommendation, even when described as "informative" or "valued", as was the case for several Australian HTAs.
- In Singapore, final recommendations were driven by cost-effectiveness and price negotiations, with negative recommendations reversed following revised pricing rather than patient input; in two reports, patient input was received but recommendations remained negative due to unfavorable cost-effectiveness.
- A notable use of patient input was in Taiwan CDE's assessment of olaparib, where a patient affordability survey (conducted by the applicant and the Taiwan Cancer Foundation) was used in the financial impact analysis. Reimbursement was subsequently granted.

Discussion and Conclusion

Real-world practice broadly reflected patient involvement guidance and mechanisms established by each jurisdiction. Australia and Taiwan leveraged online platforms to collect patient input, whereas Singapore used an invitation-based model. However, our findings suggest that providing mechanisms for patient involvement does not necessarily ensure consistent participation, particularly in rare disease assessments. Two Singaporean reports recorded no submissions received despite invitations, and nine Taiwanese reports drew on patient evidence from foreign HTA agencies, limiting the breadth or local relevance of patient input.

A further challenge relates to understanding how patient input contributes to HTA decision-making. Guidance may require documentation of what patient input was received, but not how it informed HTA recommendations. Consistent with this, the HTA reports reviewed lacked clarity on how patient input impacted HTA outcomes. Greater transparency regarding how patient input is considered alongside clinical and economic evidence could help demonstrate its contribution to decision-making.

These findings should, however, be interpreted in the context of several limitations. Our analysis was restricted to patient involvement documented in published HTA reports and may therefore underestimate the full extent of engagement across the assessment process. Activities occurring outside published reports, such as pre-PBRS meetings in Taiwan or involvement in topic nomination, were not captured. As reporting practices also differ between jurisdictions, absence of documentation is not proof of absence of patient involvement. Previous studies have reported differing extents of patient involvement across these jurisdictions,⁵⁻⁷ which may partly reflect differences in the definitions of patient involvement and differing scopes of assessment.

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