HTA27

Advocating from the Margins: How Patient Input Aligns with Broader Evidence Tolerance in HTA within Oncology

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Background

- HTA bodies increasingly acknowledge the importance of patient input in their assessments, yet its explicit weighting in decision-making remains unclear.[1]
- In oncology—where clinical uncertainty is high and treatment costs are high—patient perspectives can shape acceptability of evidence beyond hard endpoints.[2,3]
- Structural variation across HTA systems determines the level of formal engagement:
  - NICE (England & Wales),[4] PBAC (Australia)[5], and HAS (France) offer institutionalised patient pathways.[6]
  - G-BA (Germany) limits patient groups to observer status; no procedural influence.[7]
- The relationship between patient input and evidentiary acceptability —particularly in the use of surrogate endpoints—remains underexplored.

Objectives

1. Characterise the frequency and forms of patient involvement across NICE, PBAC, HAS, and G-BA
2. Examine associations between patient input, HTA outcomes, and acceptance of surrogate (e.g., PFS, ORR) over clinical (e.g., OS) primary endpoints in pivotal trials.
3. Evaluate whether institutionalised engagement pathways align with greater evidentiary flexibility in practice.

Methods

Step 1: Study Scope

- Retrospective analysis (2020–2024)
- HTA oncology appraisals (NSCLC & BC → high burden common cancers)
- Agencies: NICE, PBAC, HAS, G-BA
- Source: HTA-Hive database

Step 2: Variables Captured

- Patient input: presence, type
- Recommendation outcome (positive vs negative)
- Type of endpoints (clinical vs surrogate)

Step 3: Subgroup Stratifiers

- Subgroup analysis by:
  - HTA agency and outcome direction
  - Endpoint type (surrogate vs clinical)
  - Pooled cross-jurisdictional analysis

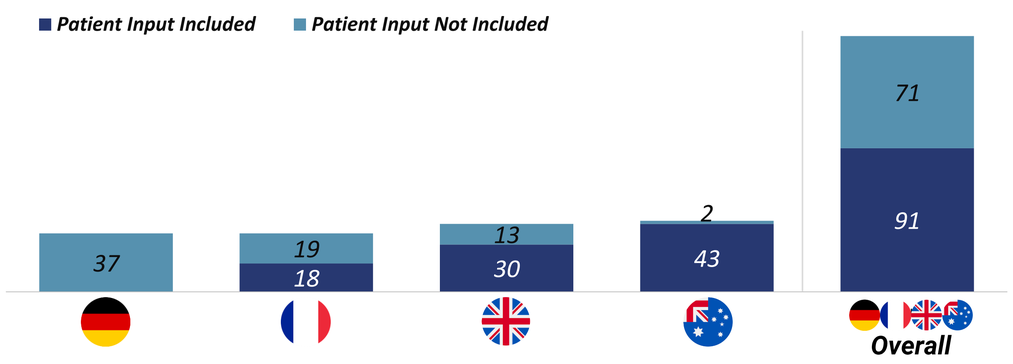
Step 4: Statistical Tests

- Fisher's Exact Test or Chi-squared tests depending on cell size
- Effect size via Cramér's V
- Odds ratios (ORs) reported for interpretability

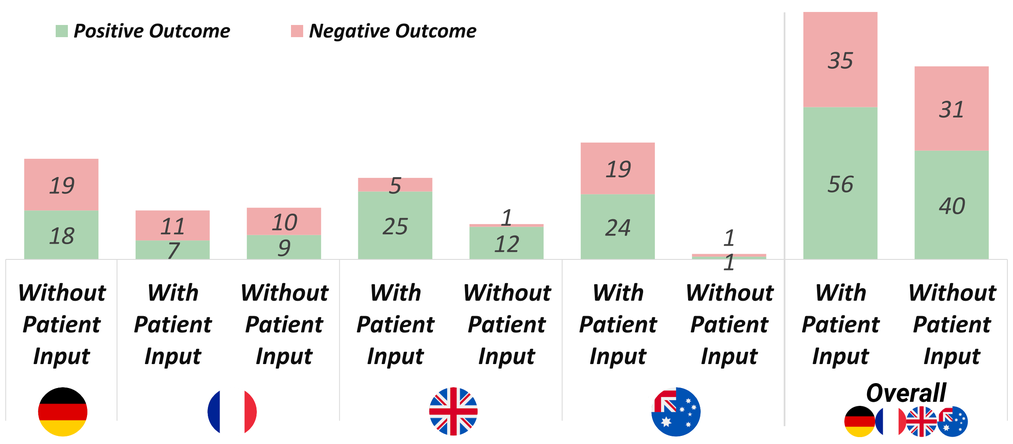
RESULTS

- A total of 162 HTA oncology appraisals were included in the analysis (2020-2024), comprising 91 non-small cell lung cancer (NSCLC) submissions (56%) and 71 Breast Cancer (BC) submissions (44%).
- The prevalence of patient input varied across agencies, with PBAC including patient input in 96% (43/45) of cases, NICE in 70% (30/43), and HAS in 44% (18/41), while G-BA included no patient input reference in their final recommendations. The main contributors of patient input were patient organisations (69%— NICE, PBAC & HAS), patient experts (NICE only) (31%), and patient individuals (PBAC only) (26%).
- No significant association was found between the presence of patient input and the final HTA recommendation. Across all three agencies—NICE (p = 0.65), PBAC (p = 1.00), and HAS (p = 0.60)—patient input was not associated with an increased likelihood of a positive HTA outcome. The pooled analysis confirmed this null effect (p = 0.74; Cramér's V = 0.03).
- Patient input was significantly more frequent when pivotal evidence relied on surrogate rather than clinical endpoints. The association was strongest in NICE, where surrogate-based appraisals were about 20 times more likely to include patient input (p = 0.0002). A suggestive trend was observed in HAS (p = 0.09; OR ≈ 7.85), with no association in PBAC (p = 1.00; OR = 0). The pooled analysis showed medium-to-large effect (p < 0.001; Cramér's V = 0.37).
- Finally, the relationship between surrogate endpoints and HTA outcomes was examined to determine whether broader evidentiary tolerance corresponded with more favourable decisions in the presence of patient input. While no significant associations were observed at the individual agency level (all p ≥ 0.2), pooled results indicated that surrogate-based appraisals involving patient input were modestly more likely to yield a positive outcome (p = 0.042; Cramér's V = 0.18).

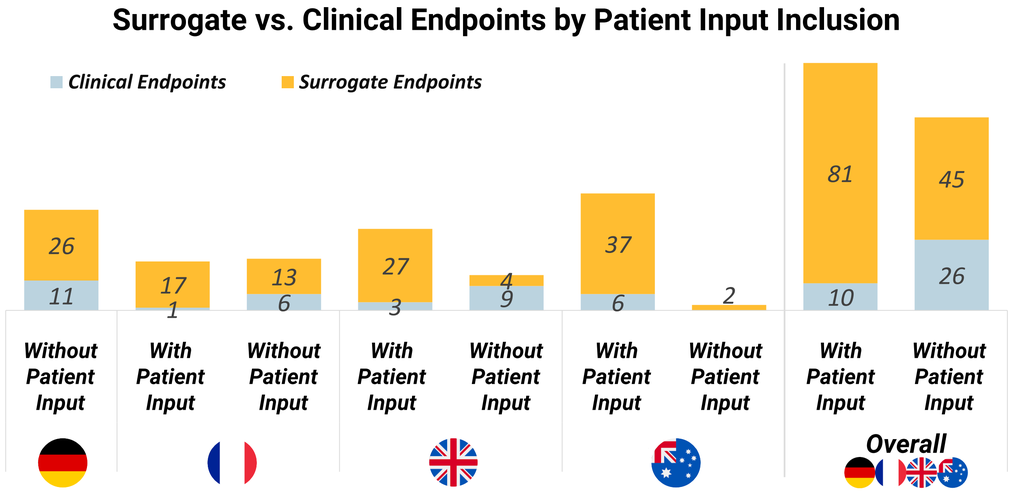
Distribution of HTA Appraisals by Presence of Patient Input



Distribution of HTA Outcomes by Patient Input Inclusion



Surrogate vs. Clinical Endpoints by Patient Input Inclusion



Conclusions

- Patient input is increasingly formalised in appraisals, but its impact on HTA outcomes remains limited. Agencies diverge considerably in how they incorporate and weigh patient input, with significant variability in frequency, influence & form of input.
- In the study sample, patient input was more common in submissions relying on surrogate endpoints, suggesting an increased use when evidentiary uncertainty is higher. However, this did not consistently translate into more favourable decisions.
- The pooled analysis suggests a modest increase in acceptance of surrogate endpoints when patient perspectives are included; however, this flexibility is not consistent across individual agencies.
- Strengthening the methodological integration of patient input can help align stakeholder perspectives with evidence standards, particularly in high-burden diseases such as BC and NSCLC.
- Continued methodological development is needed to better align patient perspectives with evidentiary standards in HTA appraisals.

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Abbreviations

Health Technology Assessment, HTA; National Institute for Health and Care Excellence, NICE; Pharmaceutical Benefits Advisory Committee, PBAC; Gemeinsamer Bundesausschuss (Federal Joint Committee), G-BA; Haute Autorité de Santé (French National Authority for Health), HAS; Progression-Free Survival, PFS; Objective Response Rate, ORR; Overall Survival, OS; Non-Small Cell Lung Cancer, NSCLC; Breast Cancer, BC

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