When Therapies are Targeted but Methods are Guessing:
Closing the Gap in Precision Oncology Evaluation

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KEY MESSAGE

Traditional economic evaluation methods are insufficient for precision oncology. A targeted review analyzing 11 recent publications reveals fragmented evaluations overly reliant on single ICERs and highlights a lack of operational HTA frameworks to guide decision-making. Flexible, pathway-based evaluation and reimbursement frameworks integrating diagnostics, real-world evidence, and multi-dimensional value assessment are urgently needed.

BACKGROUND

Precision oncology (PO) has transformed cancer care by enabling treatment tailored to patients' molecular profiles, supported by advances in genomic sequencing, biomarker identification, and companion diagnostics (CDs).[1] These innovations have improved outcomes in selected populations and accelerated the shift toward more individualized care.[2]

However, PO also exposes important limitations in health technology assessment (HTA) and economic evaluation (EE). Conventional HTA methods were developed for broader, more stable treatment populations, and may not adequately reflect biomarker-defined subgroups, linked test-treatment pathways, or rapidly evolving evidence.[3]

Key Challenges:

- **Fragmented evaluation approaches** that fail to capture the full clinical pathway
- **Limited consideration** of diagnostic accuracy, patient stratification, and treatment sequencing
- **Disconnect between** scientific advances and reimbursement frameworks
- **Underassessment of** upstream (testing infrastructure, biomarker identification) and downstream (treatment switching, resistance, re-testing) pathway components

OBJECTIVES

1. **Review** the recent methodological and policy recommendations for improving EE in PO
2. **Examine** how current frameworks define value beyond cost-effectiveness analysis/budget impact analysis (CEA/BIA)
3. **Assess** whether existing approaches reflect clinical reality and support reassessment

METHODS

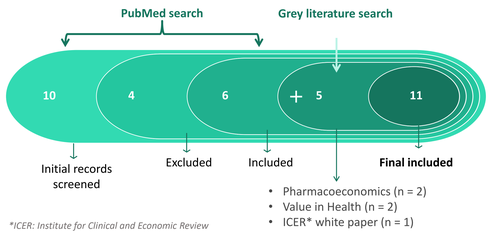
Study Design and Selection Criteria

A targeted review of EE for PO frameworks and methods (2023–2025) was conducted. **Figure 1** summarizes the study selection method.

Inclusion criteria:
- Reviews of EE frameworks or methods
- HTA publications in PO

Exclusion criteria:
- Individual trial-based CEA/BIA without methodological focus
- Non-English publications
- Pre-2023 publications

Figure 1. Study selection process



ICER: Institute for Clinical and Economic Review

Titles and abstracts were screened against predefined eligibility criteria, followed by full-text review. Study selection and data extraction were conducted using a predefined extraction grid.

Data Extraction Grid

CLINICAL/MODEL SCOPE
- Disease and population
- Analytical approach and CDs modelling

EVIDENCE INPUTS
- Real-world evidence (RWE) and external data
- Uncertainty handling

DECISION CONTEXT
- Framework type
- Reimbursement/regulatory-HTA alignment

RESULTS

11 Publications Included

3 Study Categories

Over-reliance on **ICERs** Observed in 10/11 Studies

Publication Categories

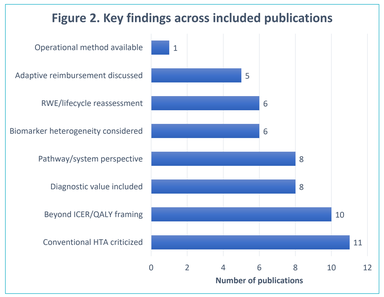
Conceptual Reviews[4-6] n=3
- Broad framework perspective
- Expands value beyond incremental cost-effectiveness ratios (ICERs)
- Highlights diagnostic, equity and system factors

HTA and EE Reviews[7-10] n=4
- Reviews current PO evaluation methods
- Critiques conventional cost-utility approaches
- Calls for more adaptive assessment

Policy reviews/documents[11-14] n=4
- Focus on reimbursement and implementation
- Emphasize access, affordability and readiness
- Mostly recommendation-based, not operational

Overall, these publications show broad agreement on the limitations of current methods, but little operational guidance for implementation in PO.

Figure 2. Key findings across included publications



What the review shows (Figure 2)

What is clear?
Current HTA approaches are widely seen as poorly aligned with the realities of PO.

What is emerging?
The literature increasingly recognizes broader value dimensions, including diagnostics, pathway complexity, biomarker-driven heterogeneity, and reassessment over time.

What is missing?
Few publications provide operational evaluation frameworks, highlighting a persistent gap between methodological critique and practical implementation.

Cross-Cutting Key Findings

1. Fragmented evaluation[7,9,11-14]
Single-ICER outputs dominate and often fail to capture pathway-level and system-level value.

2. Diagnostics under-modeled[4-7]
Diagnostic pathways, repeat testing, failure rates, delays, and the dynamic role of CDs are insufficiently represented.

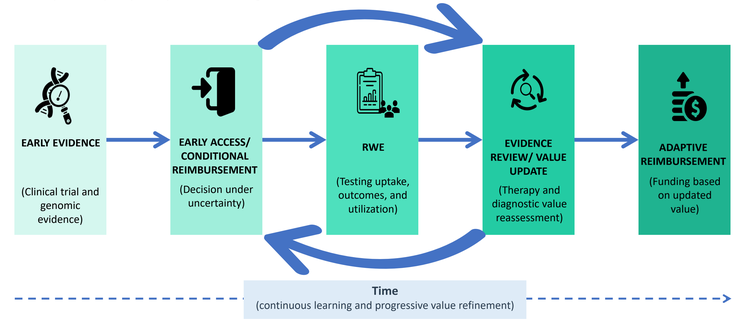
3. Real-world complexity and uncertainty[8-11]
Sequencing, heterogeneity, implementation constraints, and uncertainty remain underexplored, with limited use of advanced methods.

4. Need for broader and adaptive HTA[5,12-14]
Calls for cost-consequence analysis, multi-criteria decision analysis (MCDA), adaptive reimbursement, and closer alignment between regulatory and HTA evidence requirements are increasing.

DISCUSSION

Figure 3. Proposed direction for PO in HTA

Authors-adapted conceptual pathway based on recurring themes in the reviewed literature.[7,9,11,12,14]



A central finding of the review is that single-ICER approaches do not adequately capture the multidimensional value of PO. Broader frameworks, including cost-consequence analysis and MCDA, may provide more decision-relevant assessment.[5,7,9]

- **Methodological gap:** Single-ICER approaches are often too narrow for PO, where value is shaped by diagnostics, stratification, sequencing, and pathway-level effects.[7,9,13]
- **Policy mismatch:** Accelerated regulatory approvals are often not matched by HTA-ready comparative and cost-effectiveness evidence.[11,12]
- **Reimbursement implication:** These conditions strengthen the case for managed entry, staged access, and iterative reassessment.[5,11,14]
- **Implementation gap:** Operational factors and the use of RWE remain insufficiently standardized and incorporated into current evaluation approaches.[8-10]

CONCLUSIONS

- Traditional EE methods are insufficient for PO, with few operational HTA frameworks available[7,9,13]
- Future frameworks should adopt a pathway-based perspective, integrating diagnostics and therapeutics within a unified analytical structure.[5,11,12] **Figure 3** presents a conceptual illustration of this approach for PO in HTA.
- Critical need to incorporate RWE, account for biomarker-driven heterogeneity, and apply adaptive modeling approaches[8-10]
- Moving beyond single-metric decision-making toward multi-dimensional value assessment is essential[5,7,9]
- Greater alignment between regulators, HTA bodies, and payers is required, supported by innovative reimbursement mechanisms[11,12,14]
- This review synthesizes recent methodological and policy literature into practical themes to guide future HTA framework development in PO

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