WHITE PAPER

Integrating Real-World Evidence, Patient-Reported Outcomes, and Equity in Lifecycle HTA

Advancing Dynamic Value Assessment

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Introduction

Health technologies are increasingly introduced into clinical practice under conditions of significant clinical and economic uncertainty. Accelerated and conditional regulatory pathways, particularly in areas such as oncology, rare diseases, and advanced therapies, often rely on immature or limited evidence at the time of approval. While these pathways enable earlier patient access to innovative treatments, they also shift a substantial portion of evidence generation to the post-launch setting, requiring decision-makers to evaluate value in the context of evolving data.

This changing evidentiary landscape has challenged traditional health technology assessment (HTA) approaches, which have historically relied on point-in-time evaluations conducted at market entry. In practice, the value of a health technology evolves as evidence matures, real-world utilization becomes clearer, and patient experience is better understood. As a result, there is growing recognition that HTA systems must move beyond single assessments toward approaches that can account for change and uncertainty over time.

Lifecycle HTA has emerged as a structured response to this need. By connecting sequential activities including early evidence planning, initial appraisal, managed access, post-launch evidence generation, and reassessment, lifecycle HTA enables value to be evaluated iteratively rather than at a single time point. Within this framework, real-world evidence (RWE), patient-reported outcomes (PROs), and broader equity considerations play a critical role in reducing uncertainty and informing how value is refined across the lifecycle of a technology.

However, despite increasing recognition of their importance, the integration of real-world evidence, patient-reported outcomes, and equity-driven evidence into HTA decision-making remains inconsistent and often fragmented.

This article examines how lifecycle HTA is evolving as a decision framework, and how the integration of real-world evidence, patient-reported outcomes, and equity considerations can enable more robust, adaptive, and patient-centered value assessment over time.

Lifecycle HTA as a Coordinated Decision Framework

Lifecycle HTA represents a shift from isolated assessments toward a coordinated and adaptive decision-making framework that explicitly addresses changes in evidence, technology, and clinical context over time.

Rather than functioning as standalone evaluations, lifecycle HTA connects multiple stages of assessment into a prospectively designed sequence. A defining feature of this approach is the recognition that uncertainty at launch is not eliminated but actively managed through structured evidence generation and iterative reassessment.

This evolution fundamentally changes the role of evidence. Evidence is no longer required solely to support initial appraisal; it must be designed to inform decision points across the lifecycle, including continuation, restriction, or discontinuation decisions.

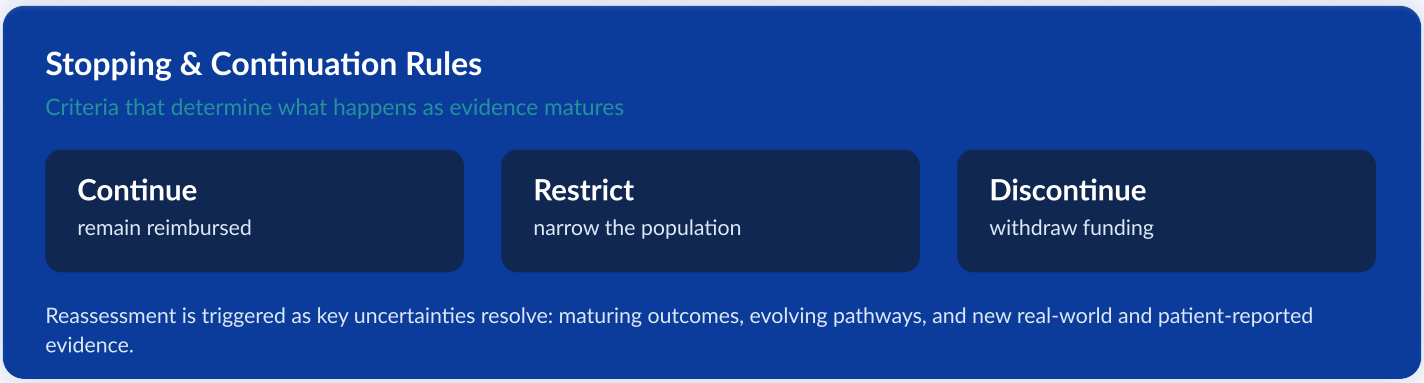
Operationalizing Lifecycle HTA: Early Access and Reassessment Pathways

Across jurisdictions, lifecycle HTA is being operationalized through early and conditional access pathways linked to reassessment, though systems sit at different stages of maturity. Early access frameworks let patients reach promising therapies on preliminary evidence while requiring prospective real-world data generation under structured evidence requirements and predefined reassessment conditions.

In France, HAS early access programs allow use on preliminary evidence while requiring structured real-world data collection to support later assessment. In the United Kingdom, NICE's Early Value Assessment aligns evidence generation with future decision needs, while managed access agreements such as the Cancer Drugs Fund enable conditional access alongside post-launch evidence generation.

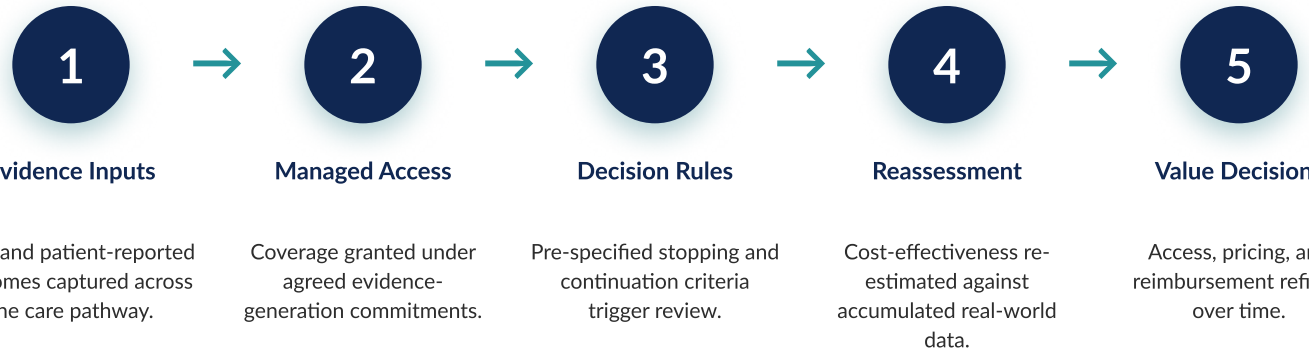
THE EMERGING PARADIGM

- Initial decisions are provisional, not definitive.
- Evidence generation is prospectively aligned with decision needs.
- Reassessment is embedded as a core component of value assessment.



From Evidence Generation to Decision-Relevant Integration

A lifecycle approach only creates value if post-launch data is translated into decisions. The pathway below connects real-world evidence and patient-reported outcomes to the access and reimbursement decisions they inform, turning ongoing data collection into structured, decision-relevant review rather than isolated analyses.



Real-World Evidence: Addressing Uncertainty Across the Lifecycle

RWE, derived from sources such as registries, administrative datasets, and electronic health records, plays a central role in lifecycle HTA. Its purpose is not merely collection but systematic translation into inputs that guide reimbursement, pricing, and access decisions over time.

RWE SUPPORTS

- Evaluation of effectiveness and safety in broader populations
- Assessment of long-term outcomes and durability
- Understanding of real-world utilization patterns
- Inputs for economic evaluation

In practice, RWE is generated through layered strategies that combine registries, administrative datasets, and observational studies alongside clinical trial extensions. Its use is highly context dependent.

RWE by Therapeutic Area

How evidence sources shift with the setting

Oncology

Registries and administrative data.

Rare diseases

Observational and registry-based evidence supplementing limited trials.

Other therapeutic areas

Targeted epidemiological and utilization data.

Real-world evidence is frequently used to contextualize uncertainty rather than replace trial evidence at initial appraisal. Within lifecycle HTA, its role is expanding toward informing reassessment decisions and updating value over time.

Patient-Reported Outcomes: Capturing Patient-Centered Value

PROs provide direct insight into the patient experience, capturing dimensions of value not fully reflected in clinical endpoints, including quality of life, symptom burden, and treatment impact.

These outcomes are particularly relevant in chronic conditions, rare diseases, and settings with limited clinical endpoints. While widely recognized, they are often incorporated in a qualitative or supportive capacity (for example, descriptive quality-of-life outcomes, caregiver burden assessments, or utility inputs used within economic models) rather than as formal components of decision-making frameworks.

A MORE CENTRAL ROLE

- Informing value judgments when clinical outcomes are uncertain
- Supporting continuation or discontinuation decisions
- Providing evidence of patient benefit beyond traditional endpoints

Realizing this potential requires systematic integration into clinical, economic, and RWE frameworks, ensuring that patient experience is meaningfully reflected in value assessment.

Equity as a Core Dimension of Lifecycle Value

Equity considerations are increasingly recognized as a critical component of value assessment within lifecycle HTA.

THESE CONSIDERATIONS INCLUDE

- Patient and caregiver burden
- Real-world access and treatment complexity
- Population-level disparities in access and outcomes

RWE and PROs provide the foundation for assessing these dimensions. RWE identifies disparities in access and outcomes, such as subgroup differences in uptake across socioeconomic or geographic groups, while PROs capture variation in treatment burden and lived experience, such as differences in quality of life or tolerability across populations.

However, equity considerations remain largely qualitative. Advancing lifecycle HTA will require their systematic and data-driven integration into decision-making frameworks.

The Integration Imperative

The effectiveness of lifecycle HTA ultimately depends on the integration of evidence within decision-making processes. Currently, RWE informs effectiveness and utilization, PROs inform patient experience, and equity considerations provide contextual insight. However, these elements are often assessed in parallel rather than within a unified framework.

INTEGRATED EVIDENCE SYSTEMS, WHERE

- Data generation is aligned with clearly defined decision questions
- Clinical, economic, and patient-centered outcomes are connected
- Evidence evolves alongside technologies and their real-world use

This shift requires evidence to be treated as a connected system rather than a set of parallel inputs, so that each element reinforces the others across the lifecycle.

Without this integration, lifecycle HTA risks being structurally robust but analytically constrained.

Implications for Market Access and Health Economics

The transition to lifecycle HTA is reshaping how evidence is generated and used in market access decisions. Market access is no longer a discrete event at launch; it is an ongoing, lifecycle process requiring early alignment between clinical development, evidence strategy, and payer expectations.

This is reflected in recent appraisals. Pembrolizumab-based combinations in metastatic lung cancer progressed through Cancer Drugs Fund exit review, with updated survival evidence from KEYNOTE-407 and real-world data sources informing routine commissioning. Atezolizumab in the adjuvant setting underwent managed access review, with evidence generated under managed access agreements supporting guidance updates and long-term reimbursement.

Evidence planning must extend beyond initial appraisal to anticipate the uncertainties that drive reassessment. At the same time, static reimbursement models are giving way to adaptive frameworks where access and pricing are conditionally linked to evidence maturation.

HEALTH ECONOMIC EVALUATION, IN PRACTICE

- Updating survival estimates and durability as long-term data mature
- Refining assumptions on treatment duration, adherence, and real-world use
- Incorporating comparative effectiveness evidence from real-world data
- Integrating patient-reported outcomes to reflect quality-of-life impacts
- Accounting for changes in comparators, treatment pathways, and market dynamics

Rather than full re-appraisal, these approaches focus on targeted parameter updates, producing more robust and mature estimates of cost-effectiveness and signaling a shift toward a dynamic, evidence-responsive health economic paradigm.

Conclusion

Lifecycle HTA represents a fundamental shift in how healthcare systems evaluate, manage, and refine value over time. It moves beyond static, one-time assessments toward an adaptive and evidence-responsive model in which decisions are continuously revisited as new evidence emerges.

Within this framework, structured reassessment and ongoing evidence generation are integral to decision-making. Early access, managed evidence generation, and iterative evaluation enable healthcare systems to accommodate uncertainty while maintaining accountability in value determination.

RWE, PROs, and equity are not supplementary inputs, but core components of lifecycle HTA.

Their effective integration into decision frameworks will determine how successfully this approach supports adaptive, patient-centered, and sustainable healthcare systems.

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