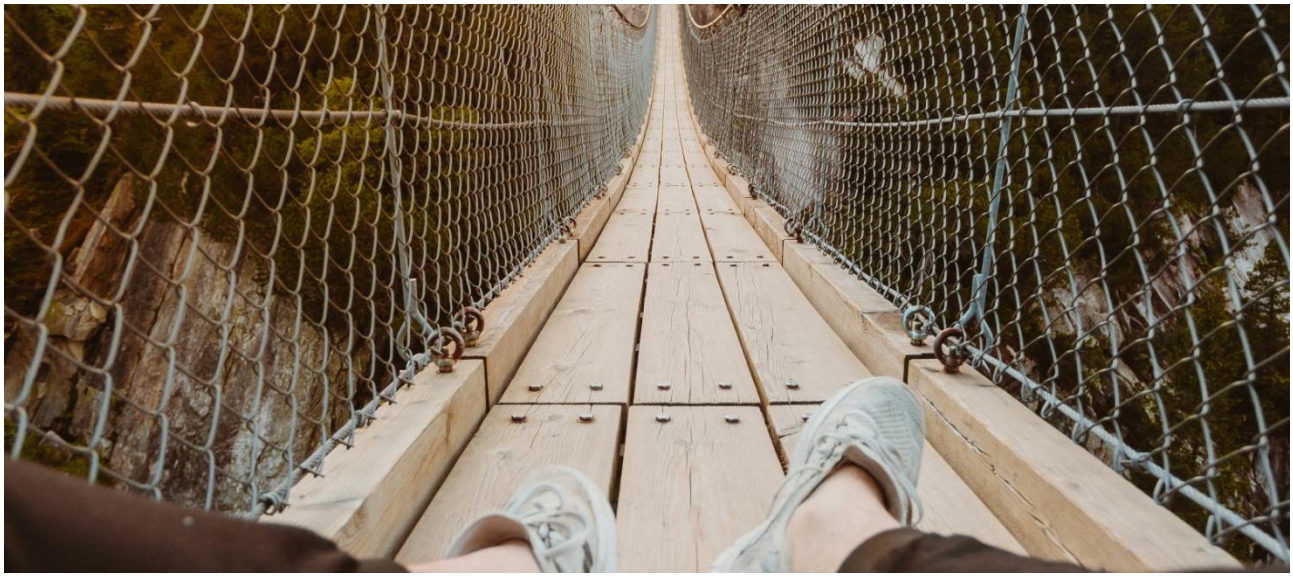
executive insight
HEALTHCARE CONSULTANTS

Closing the Patient Evidence Gap:

Translating Patient Experience into Decision-Relevant Evidence for HTA, Payers, and Patient Access



Introduction

The next frontier in evidence generation is ensuring that evidence strategies and investments are guided by the decisions that matter most for reimbursement, access, and patient-relevant value. For many years, Patient Experience Data (PED) played a relatively minor role in evidence planning, often remaining adjacent to core clinical, HEOR, access, and medical evidence activities. Regulatory expectations have started to change this, with initiatives such as FDA's Patient-Focused Drug Development and EMA's recent reflection paper on PED helping to legitimize patient experience as an important component of drug development and benefit-risk thinking.

Across the product lifecycle, patient evidence can inform decisions from healthcare policy to regulatory, access, uptake, care delivery and real-world use. This article focuses on HTA, payer decision-making, reimbursement, and patient access because this is where

patient evidence is increasingly expected to support value assessment, address uncertainty, and demonstrate real-world relevance.

This is where the role of PED is set to grow. HTA bodies and payers increasingly need to understand not only whether a drug works, but how it changes daily life, reduces burden, supports functioning, and delivers outcomes that patients recognize as meaningful. To influence these decisions, patient evidence must be robust, systematic, and embedded early within Integrated Evidence Planning (IEP). Yet today, IEP is often still organized around functional evidence activities rather than stakeholder decisions. Clinical Development, Market Access, HEOR, and Medical Affairs usually drive the process, while Patient Affairs is often not directly involved or, at best, provides input into selected activities.

We refer to this as the **Patient Evidence Gap**: the disconnect between the growing recognition of patient experience as strategically important and its limited integration into formal evidence planning and investment decisions. Closing this gap matters because it connects patient relevance with decision relevance, helping organizations generate evidence that can support value assessment, reimbursement, and access for those most likely to benefit.

The Patient Evidence Gap

The industry has made substantial progress in incorporating the patient perspective into research, development, and regulatory decision-making. Yet collecting patient insights and generating decision-relevant patient evidence are not the same.

Many organizations gather patient perspectives through advisory boards, interviews, journey mapping, or engagement activities. Far fewer translate these insights early into evidence strategies that can inform HTA, payer, reimbursement, and access decisions. Too often, PED is addressed reactively, once evidence gaps have already become visible in regulatory, HTA, or access discussions. This leaves a gap between what patients say matters and what evidence portfolios are designed to prove.

To close this gap, PED needs to be understood not only as a source of patient insight, but as a strategic evidence domain that can inform access-relevant decisions from the earliest stages of planning.

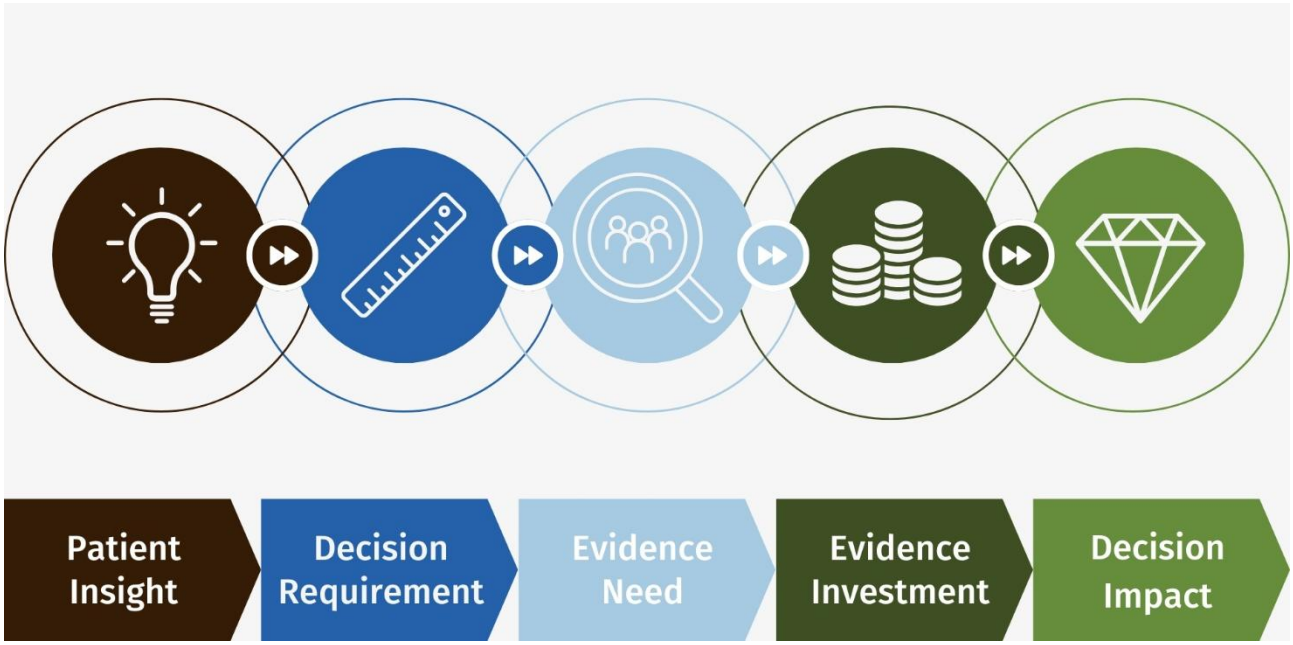
Reframing Patient Experience Data

Patient experience data refers to information collected directly from patients, caregivers, or patient organizations about their lived experiences with a disease. It captures their perspectives, priorities, and preferences regarding symptoms, treatment impact, quality of life and outcomes that matter most to them. Within IEP, the central question from a patient access perspective should be: "What patient evidence is needed to support HTA, payer, and access decisions?"

The Patient Evidence Gap is therefore best understood as a translation challenge: moving from patient insight to decision impact. Most organizations are increasingly successful at generating patient insights. Far fewer are successful at turning those insights into evidence investments that can influence HTA, payer, reimbursement, and access decisions.

The framework below shows how patient insight can be systematically translated into evidence choices with a clearer line of sight to access impact.

The Patient Evidence Translation Framework:



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Step	Key question	Output
Patient Insight	What matters to patients?	Unmet needs, burdens, preferences, and outcomes that matter most
Decision Requirement	What must HTA bodies and payers decide?	Key value drivers, uncertainties, and evidence expectations
Evidence Need	What must be proven?	Evidence gaps, endpoint implications, and priority hypotheses
Evidence Investment	What evidence should be generated?	PROs, preference studies, patient experience research, burden evidence, and real-world evidence
Decision Impact	How will this influence access?	Improved value assessment, reimbursement decisions, and patient access

For example, if patients consistently report that fatigue has a greater impact on daily life than clinicians appreciate, the insight alone will rarely influence reimbursement or access. It becomes decision-relevant only when translated into an evidence need: whether fatigue represents a meaningful uncertainty for HTA bodies or payers, whether improvement can be measured robustly, and whether that evidence could strengthen the value story. The resulting investment may include PROs, preference research, qualitative studies, or real-world evidence designed to support future access decisions.

PED as a Strategic Evidence Domain within Decision-Led IEP

Integrated Evidence Plans define what evidence is needed, why it is needed, and when it should be generated across the product lifecycle. In a decision-led IEP approach, the starting point is not a list of studies, but the stakeholder decisions that will shape access, reimbursement, uptake, and patient impact.

This is where PED expertise adds a distinctive lens. Patient Affairs teams understand what patients consider meaningful, how disease and treatment affect daily life, and where patient value may not be captured by traditional clinical or economic measures. Within IEP, this expertise should inform evidence priorities, endpoint implications, real-world evidence needs, and investment trade-offs.

Five patient evidence domains are particularly relevant for HTA and payer decisions:

1. **Unmet need evidence:** disease burden, care pathway challenges, and gaps in available treatment options.
2. **Experience evidence:** impact of disease and treatment on daily life, functioning, and quality of life.
3. **Preference evidence:** benefit-risk trade-offs, treatment preferences, and what patients value most.
4. **Value evidence:** patient-relevant outcomes, caregiver impact, societal benefits, and evidence supporting access arguments.
5. **Implementation evidence:** treatment adoption, adherence, persistence, and real-world use.

Together, these domains create the basis for evidence investment trade-offs: what to prioritize, what to pause or stop, and where patient evidence could strengthen the access case.

Quantifying the Patient Evidence Gap: What HTA Reports Suggest

Because company IEPs are rarely public, HTA submissions and assessment reports offer a useful external lens into whether patient evidence is becoming decision-relevant. Recent reviews of NICE and CADTH submissions, NICE final appraisal documents, and highly specialized technology assessments show a consistent pattern: patient input is increasingly present, but it is not always generated, analyzed, or reported in ways that make it credible as decision-shaping evidence.

This pattern is visible across both rare and more common disease contexts, but the evidence challenge differs. In rare and ultra-rare diseases, patient evidence has historically been more prominent because small populations, limited comparative data, and high uncertainty make lived experience an important source of context. In more common chronic diseases, the challenge is often different: patient evidence may be available, but needs to be more comparative, quantified, and explicitly linked to value conclusions to influence HTA and payer decisions.

Evidence box: Rare versus common disease patterns in HTA use of patient evidence

Dimension	Rare / ultra-rare diseases	Common / broader chronic diseases
Visibility of patient voice	Patient input is often highly visible and valued for contextualizing unmet need, burden, disease severity, and uncertainty. In one NICE review, all rare disease final appraisal documents recorded patient input.	Patient input is often present, but may be less differentiated or less influential in final value assessment. In a NICE review of 158 final appraisal documents, 87% mentioned patient input, but its role in shaping conclusions varied.
Type of contribution	Often qualitative and contextual, helping committees interpret limited evidence, small trials, caregiver burden, and lived experience. In highly specialized technology assessments, no respondents stated that patient input had no impact, compared with 35% in interventional procedure assessments.	Often supportive rather than decision-shaping unless linked to robust PROs, HRQoL, treatment burden, preference evidence, or comparative outcomes. Across NICE appraisals, patient input most often related to QoL (60%), unmet need (43%), and clinical outcomes (42%).

Typical evidence gap	Patient voice is central, but may not always be generated, analyzed, or reported with sufficient methodological rigor. In the NICE review, none of the rare disease appraisals used quantitative patient input, despite all recording patient input.	Patient perspectives may be captured, but not sufficiently quantified, differentiated, or connected to the endpoints and value arguments that drive HTA decisions. Only 4% of NICE appraisals used quantitative patient input; in oncology, only one final appraisal document mentioned quantitative collection of patient experience.
IEP implication	Plan PED early to strengthen credibility, structure qualitative evidence, and make lived experience usable in uncertainty-based assessments.	Plan PED early to generate decision-relevant, comparative patient evidence that can influence value assessment alongside clinical and economic evidence.

Across both rare and common disease contexts, the underlying challenge is the same: patient perspectives are being heard more often, but they do not yet consistently translate into evidence that changes the assessment of value. From an IEP perspective, this represents a form of value leakage: patient insights are generated and discussed, but not systematically translated into decision-relevant evidence that can influence stakeholder decisions on value, reimbursement, and access. Closing this gap requires planning PED early with the access decision in mind: in rare diseases, by making lived experience more systematic and credible; in common diseases, by making patient evidence more comparative, quantified, and connected to value arguments.

Looking Ahead: From Patient Insight to Access Impact

For PED, the future opportunity is clear: move beyond engagement and insight generation toward earlier, more deliberate evidence planning, with a clear line of sight between what patients value, what decision makers need to understand, and which evidence investments could shift access outcomes.

Closing the Patient Evidence Gap requires more than generating patient insights. It requires translating those insights into evidence that can shape value assessment, guide access decisions, and ultimately determine whether innovations reach the patients who need them most. In a decision-led IEP, PED becomes more than a source of patient insight; it becomes a strategic lever for demonstrating value, reducing uncertainty, and ensuring that access decisions reflect what patients experience, need, and value most.

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