Robust Use of Patient Experience Data to Inform Regulatory Decision-making and Labeling

BACKGROUND AND CONTEXT

On May 6-7, 2026, the Duke Clinical Research Institute convened an interdisciplinary Think Tank, bringing together regulators, sponsors, methodologists, payers, academics, and patient advocacy representatives to examine how **patient experience data (PED)** can be generated, interpreted, and applied more effectively and efficiently across the medical product lifecycle. The group affirmed PED's strong potential for evidence generation and decision-making at every stage of drug development and identified key opportunities to address barriers related to planning, evidentiary expectations, and accountability for the collection and use of PED.

MAJOR TAKEAWAYS

Early and intentional planning to build the patient narrative that informs decision-making

- **PED should be integrated into the clinical development plan as early as possible,** with clearly articulated intended use cases across development through post-market monitoring. Multidisciplinary input, including early and iterative engagement with regulators, is critical to align evidence expectations and communicate intended use to support benefit-risk assessments and broader regulatory decision-making.
- **No single method fits every context.** Mixed-methods (qualitative and quantitative) approaches are often needed to capture the full impact of disease and treatment. This may include independent or collective insights from qualitative studies, patient-reported outcomes (PROs), and patient preference information (PPI) studies.
- **PED comes directly from patients, capturing how patients feel, function, and weigh treatment decisions in ways third-party measures may not fully reflect.** Concerns about PED, such as subjectivity in PROs and hypothetical bias in PPI, should be carefully addressed, but weighed against their demonstrated value. For example, PROs can surface symptoms that clinicians may otherwise underreport, while PPI instruments undergo steps of rigorous development and validation to quantify benefit-risk trade-offs.

The importance of "decision-grade" evidence

- **The evidentiary bar for PED should match the decision it is intended to support, with regulatory approval being *only* one of several decision points.** Beyond regulatory approval, products also need to withstand payer coverage, health technology ssessment (HTA) decisions, and real-world adoption, often across multiple jurisdictions with differing evidentiary expectations. PED should therefore be planned across the full arc of evidence needs, not for a single decision point.
- **Greater global harmonization across regulators, HTA bodies, and payers** is needed to reduce duplicative evidence requirements and improve the quality, relevance, and efficiency of PED generation and utility.

Balancing rigor and practicality, and shared accountability to support broader PED adoption

- **Legacy endpoints and instruments** may have extensive use but are not always the most informative for a given therapeutic area. While they may serve as a practical starting point, measures lacking validity for the intended use should be adapted or developed de novo in line with existing guidance. Selection should balance feasibility, stakeholder expectations, and methodological rigor.
- **Careful attention is needed for patient burden.** Lengthy or redundant questionnaires, particularly in trials with multiple PROs, risk reducing data quality through fatigue or disengagement. Thoughtful planning is needed to optimize the order and frequency of assessments to ensure PED collection is both rigorous and feasible.
- **Despite available guidance and frameworks, stakeholders may be risk-averse to invest in or prioritize PED due to uncertainty around incentives and downstream use.** While agreement on the importance of PED appears to be growing with regulatory attention, fundamental questions remain about accountability and stakeholder roles in ensuring PED is meaningfully collected and used.
- **Regardless of trial success, the underlying science and PED collected retain value.** Data sharing can be constrained by consent forms, data ownership provisions, and publication rights that limit dissemination of PED findings. Additional barriers include the lack of upfront requirement to publish, as well as reluctance or limited resources to disseminate negative findings. However, knowledge sharing helps collectively de-risk PED generation and maximize the value of collected data.

ACTIONABLE ITEMS

- Adopt and promote the term **"decision-grade"** evidence in place of "regulatory-grade" across relevant communications and documents.
- Translate successful standardization models, such as PROs in oncology, to other therapeutic areas through **PED toolkits** with therapeutic-specific guidance. Shared learning should be advanced through **publications of qualitative, PRO and PPI results from both positive and negative trials**, supported by proactive consent language, data use agreements, and data sharing through platforms such as ClinicalTrials.gov.
- Advocate for cultural and policy change to **incentivise or require robust PED** collection across the product lifecycle, such as requiring or rewarding collection during pre- and post-market phases and after treatment discontinuation, alongside sharing examples of how PED informs decision-making.
- **Operationalize existing guidance** into practical lifecycle planning tools that extend from early development through post-market use, rather than ending at approval. This continuum includes leveraging collective meeting pathways to engage with regulators, payers, and medical societies early to align on evidence needs.
- **Convene a working group of international regulators**, HTA bodies, and payers to align on globally shared PED evidentiary expectations, starting with areas with established PRO experience such as oncology.

For more information, please visit
https://dcri.org/insights-and-news/insights/dcri-think-tanks.