Insight Brief

Patient Experience Data as Decision Grade Evidence: Regulatory Strategy Insights from EMA's Reflection Paper

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External-facing note: This Insight Brief reflects IQVIA's perspective on publicly available frameworks and consultation documents. It is intended for informational purposes and does not constitute legal or regulatory advice.

Executive summary

The European Medicines Agency's (EMA's) draft Reflection Paper on Patient Experience Data (PED) (Ref. EMA/CHMP/PRAC/148869/2025) was published for public consultation on 29 September 2025, with comments accepted through 31 January 2026. As a reflection paper issued for consultation, it does not establish binding requirements, but it does signal that patient experience evidence is expected to be planned and submitted as decision-relevant information in the European Union's medicines development. From a regulatory strategy standpoint, the opportunity now is to translate these high-level principles into clearer expectations that explicitly connect PED to regulatory decision points, use consistent scope and terminology, including the full Clinical Outcome Assessment (COA) taxonomy, and support more proactive and fit-for-purpose evidence planning while enabling greater consistency in how PED is framed across regulatory, Health Technology Assessment (HTA), and access-related evidence contexts. Over time, stronger evidence planning and documentation could also improve the reusability of PED packages across major markets, including Europe, the United States, Japan, and China, while recognizing that jurisdiction-specific adaptation will still be required.

Why this matters now

Two forces are converging. First, regulators are increasingly placing greater emphasis on systematic approaches for collecting and using patient input to inform product development and regulatory decision-making. Second, Europe's evidence environment is becoming more integrated, with Joint Clinical Assessments (JCAs) under the HTA regulation increasing the need for coordinated evidence narratives alongside marketing authorization. Together, these trends increase the value of PED that is fit-for-purpose, interpretable, and reusable across jurisdictions.

Regulatory signals and outstanding questions

The EMA's paper encourages early dialogue with regulators on how PED will be generated, analyzed, and submitted. It recognizes multiple forms of PED, including COAs, qualitative research, patient preference studies, and patient-generated digital data collected in clinical trials or in real-world research settings. At the same time, the paper leaves key questions open that directly affect evidence planning: how PED should be framed for specific regulatory decisions (beyond general encouragement), whether PED should be interpreted narrowly as Patient-Reported Outcomes (PROs) or more broadly to encompass other COA types, and what minimum fit-for-purpose expectations apply across different PED types and development stages.

Making PED decision-ready

For PED to consistently influence regulatory assessment, it must be anchored to the decision context. In practice, this means clarifying how PED informs:[1] selection and prioritization of patient-relevant endpoints;[2] interpretation of meaningful change and difference (including how thresholds are defined and justified);[3] benefit–risk assessment, including tolerability and preference-sensitive trade-offs; and[4] where appropriate, communication in product information. Clear linkage between PED and these decision points improves predictability for sponsors and helps reviewers evaluate relevance and rigor proportionately.

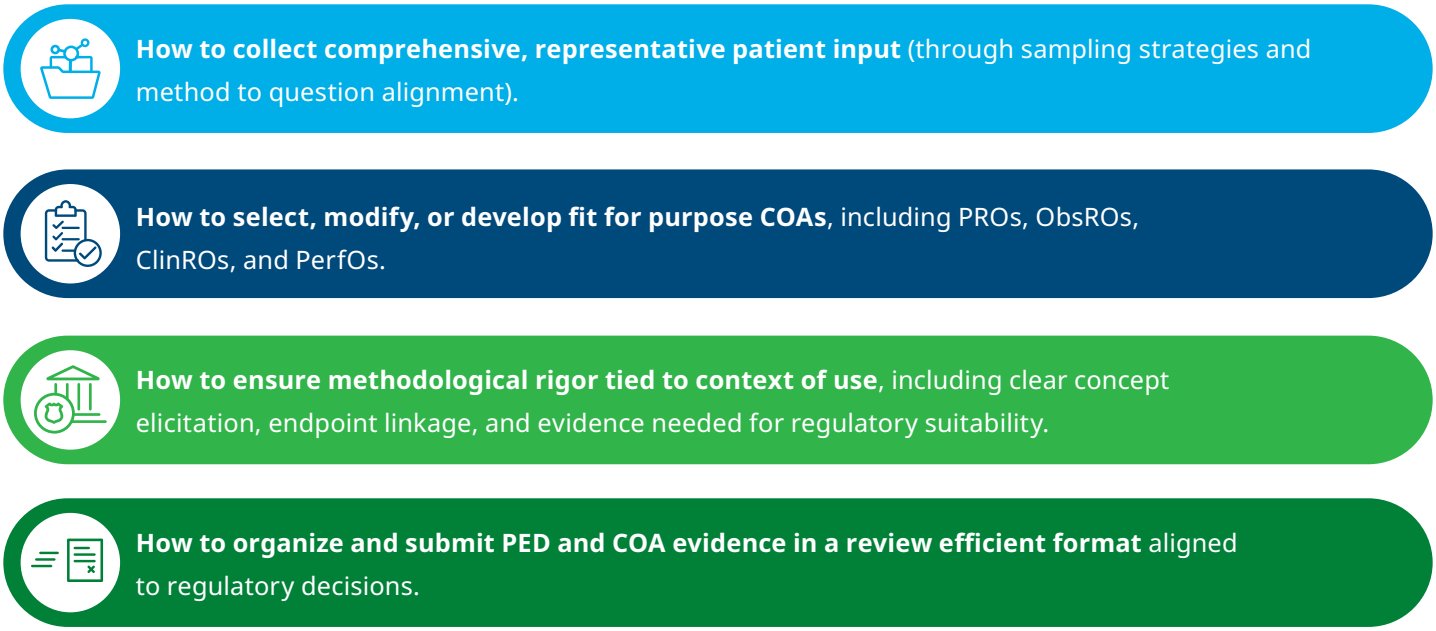
A second implication is the need for terminological precision. PED should not be interpreted as synonymous with PROs alone. A regulatory grade approach should reflect the full COA taxonomy — PROs, Observer-Reported Outcomes (ObsROs), Clinician-Reported Outcomes (ClinROs), and Performance Outcomes (PerfOs) — so evidence strategies remain inclusive of populations that cannot self report and reflect concepts that matter to patients, including when outcome-relevant data are collected passively through DHTs.

Advancing convergence in PED and COA strategy

Global alignment is increasingly important for efficient global development programs, particularly as sponsors aim to generate patient experience evidence that is interpretable, decision relevant, and portable across regulatory and HTA contexts. Three major frameworks, FDA's Patient Focused Drug Development (PFDD) guidance series, the EU HTA Regulation, and the JCA, are shaping expectations for how PED and COA evidence should be generated, organized, and reviewed. Although these pathways do not yet reflect full convergence, together they underscore the value of more coordinated evidence planning across development programs. They also provide a foundation for evidence packages that may be more readily leveraged in other important markets, including Japan and China, where interest in patient-centered evidence and more structured use of such data is continuing to evolve.

FDA's Patient-Focused Drug Development (PFDD) framework

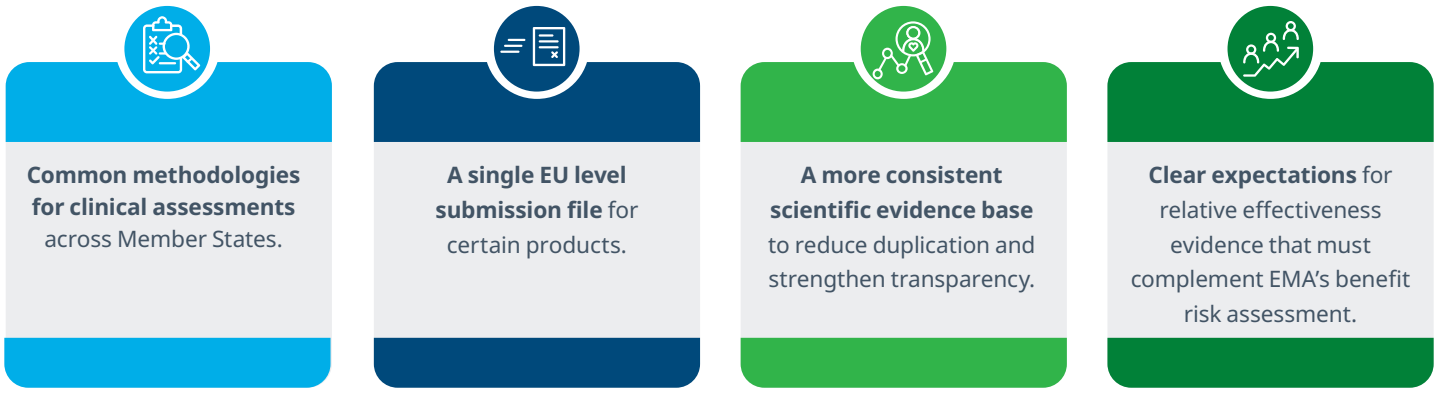
FDA's PFDD framework is the most mature global model for systematically incorporating PED into regulatory decision making. It consists of a four-part methodological guidance series that addresses:



The PFDD guidance series reinforces the importance of high-quality patient-centered measurement, discourages proxy-reported outcomes for patient experience, and provides a detailed "roadmap to patient-focused outcome measurement" to guide sponsors from early development through endpoint justification. Collectively, these elements provide a well-developed framework that can inform EMA's evolving PED expectations and support more consistent evidence planning across regions.

Health Technology Assessment (HTA) and the EU HTA regulation

Health Technology Assessment (HTA) is a multidisciplinary evaluation of a health technology's comparative clinical effectiveness, safety, economic impact, and broader ethical and social considerations. Under **Regulation (EU) 2021/2282**, the EU has established a shared HTA framework to create:



This regulatory environment places additional value on PED that clarifies patient relevant outcomes, meaningful change thresholds, tolerability, and preference sensitive tradeoffs, all of which feed into HTA bodies' comparative effectiveness evaluations and ultimately, national pricing and reimbursement decisions.

Joint Clinical Assessment (JCA) under the EU HTA regulation

The **Joint Clinical Assessment (JCA)** represents a major operational shift in Europe. Beginning January 2025, JCAs provide an EU level scientific assessment of the *relative clinical effectiveness and safety* of selected medicines. Key features include:

- **Mandatory JCAs for new oncology medicines and advanced therapy medicinal products (ATMPs)**, expanding to orphan medicines in 2028 and all new medicines in 2030.
- **Parallel review with EMA**, meaning clinical evidence must be prepared in a coordinated and timely manner.
- **A unified PICO framework** (Population, Intervention, Comparator, Outcomes) that defines evidence expectations across Member States.
- **A single EU level clinical dossier**, reducing redundant national submissions.
- **A scientific report that feeds into national HTA processes**, which remain responsible for economic evaluation and reimbursement decisions.

For PED specifically, the JCA framework amplifies the need for early alignment on patient relevant outcomes, meaningful change definitions, treatment related burden, and preference evidence. PED that is well structured and rigorously justified can support both EMA's regulatory assessment and HTA bodies' comparative analyses, improving coherence across the evidence lifecycle.

Because the EU HTA Regulation introduces JCAs for selected medicines, developers increasingly need evidence plans that can serve both regulatory and HTA decision contexts. Clearer articulation of how PED may contribute to JCA submissions, would reduce duplication and improve strategic planning.

Advancing a harmonized COA evidence dossier framework

How evidence is organized matters. FDA released a Draft COA Evidence Dossier Template in September 2025 as a discussion document to help facilitate submission of evidence in support of COAs and improve review efficiency. Importantly, the template was presented in the context of FDA's September 18–19, 2025 PFDD Workshop #2 and underwent a public comment period (Docket No. FDA-2025-N-2368; comments due 18 November 2025). Although the FDA's draft template is not a global submission format, it illustrates the value of a more structured and transparent way to organize COA evidence. Over time, greater alignment in how sponsors compile concepts of interest, instrument selection or modification, endpoint justification, and evidentiary support would help reduce rework across regulatory interactions. In the EU HTA context, that type of structured evidence organization could also help sponsors document patient relevant outcomes, meaningful change, and other COA related considerations more consistently for JCA and related HTA discussions, even if the required submission formats and decision standards remain distinct.

Patient preference evidence in a converging landscape

As regulators sharpen expectations around the role of patient preferences in benefit–risk evaluation, the field is entering a period of meaningful convergence in underlying methodological expectations. EMA's growing emphasis on incorporating patient preference studies into regulatory decision making reflects a broader international shift toward structured, methodologically defensible approaches to understanding what patients value, what trade-offs they are willing to make, and how these insights should shape regulatory judgments. In parallel, long-standing multi-stakeholder initiatives, including IMI-PREFER and the draft ICH E22 guideline, are helping establish shared principles for the design, conduct, and analysis of preference studies intended for regulatory use.

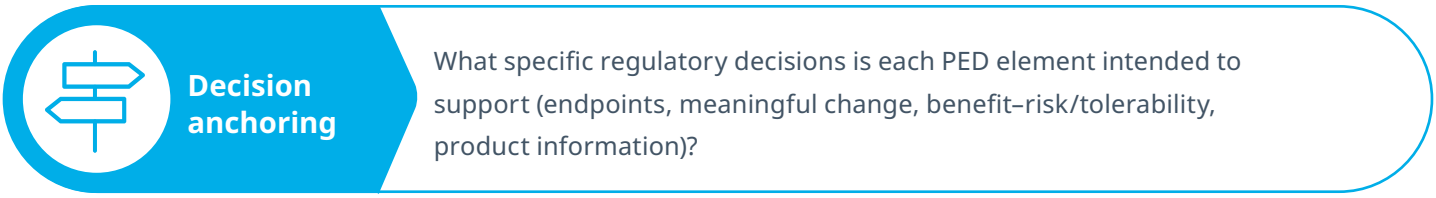
This broader movement matters for sponsors because it increases the likelihood that patient preference evidence will be treated as a more decision-relevant evidentiary stream. IMI-PREFER provides practical recommendations for when preference studies are most informative, how to structure them to capture preference heterogeneity, and how to ensure that data collection and analysis methods match the regulatory question at hand. ICH E22 builds on this by offering the first international consensus on core concepts, such as defining the decision context, selecting appropriate elicitation methods, ensuring representativeness, and transparently documenting analytic choices. Together, these frameworks help reduce ambiguity around what "regulatory-grade" preference evidence should look like.

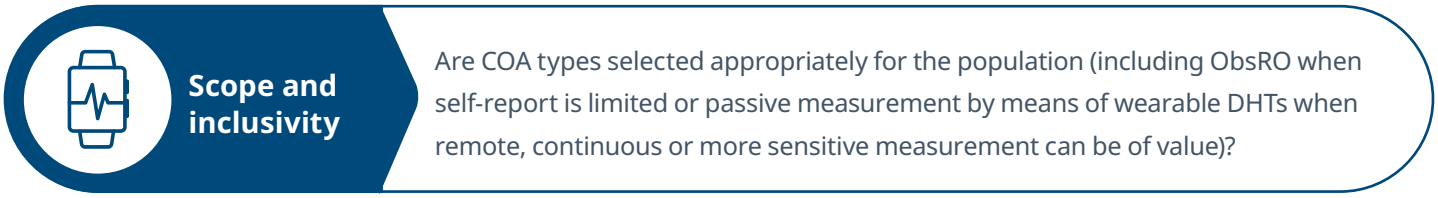
From a strategic perspective, the opportunity is to connect EMA's evolving PED expectations with these established methodological foundations. Doing so could create clearer routes for sponsors to generate preference evidence that is more readily interpretable across EMA, FDA, and HTA settings to support coherent benefit–risk narratives, inform endpoint selection where patient-valued outcomes differ from historical norms, and clarify acceptable trade-offs for tolerability, administration burden, or therapeutic uncertainty. Importantly, more consistent approaches could also reduce unnecessary rework for global programs and help ensure that preference studies are designed with broader downstream utility in mind across regulatory, clinical, and access decision points.

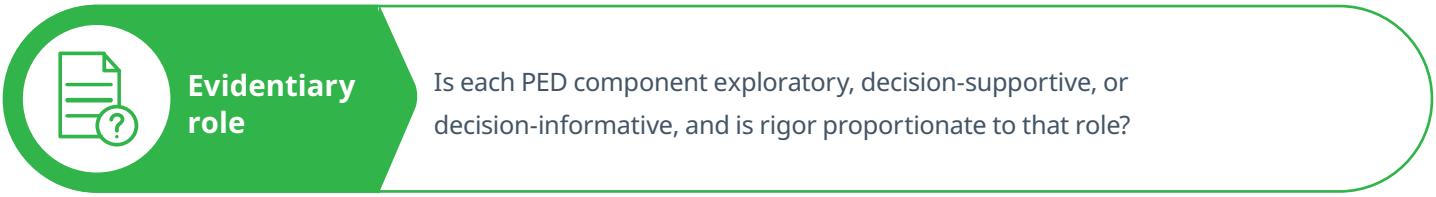
In this environment, the strategic question for sponsors is no longer whether to integrate patient preference evidence, but *how to plan it proactively* so that it aligns with emerging global standards. Anchoring preference work in IMI-PREFER and ICH E22 from the outset, while actively monitoring EMA's evolving expectations, can help ensure that evidence is methodologically robust and usable across multiple regulatory and HTA settings, strengthening its impact across development and access pathways.

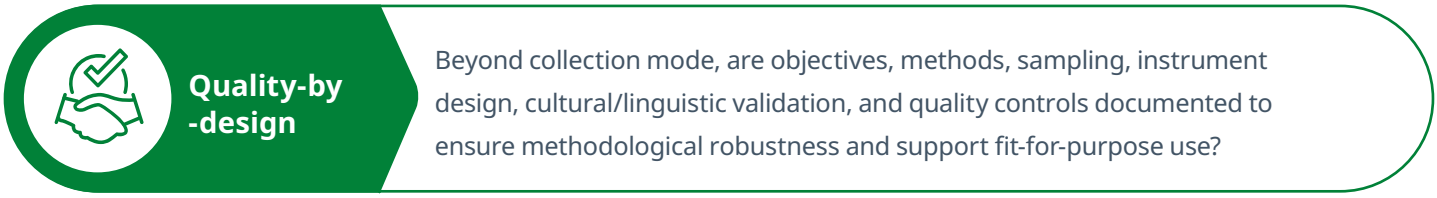
A practical framework for PED planning

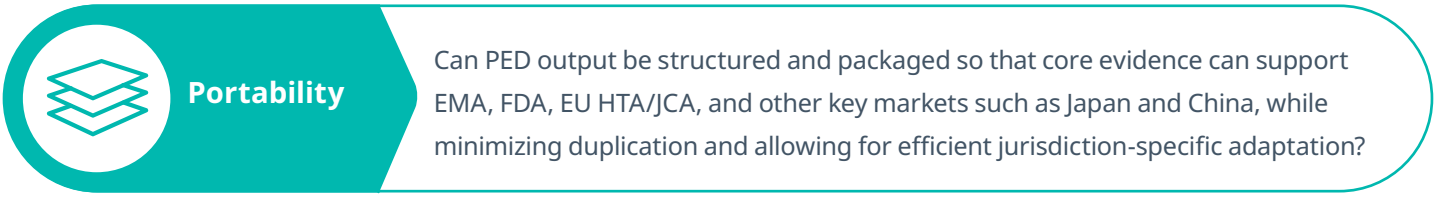
As regulatory expectations for PED become more structured across EMA, FDA, and EU HTA bodies, sponsors increasingly need a practical way to translate these high level principles into day to day evidence planning decisions. The following prompts are designed to operationalize the concepts described in the preceding sections, helping teams quickly anchor PED generation to specific regulatory and HTA decision points, ensure methodological proportionality, and anticipate cross regional evidence requirements. Rather than prescribing a single approach, these considerations support internal alignment and early strategic dialogue so that PED is consistently planned, justified, and packaged in a way that is decision ready across global pathways.

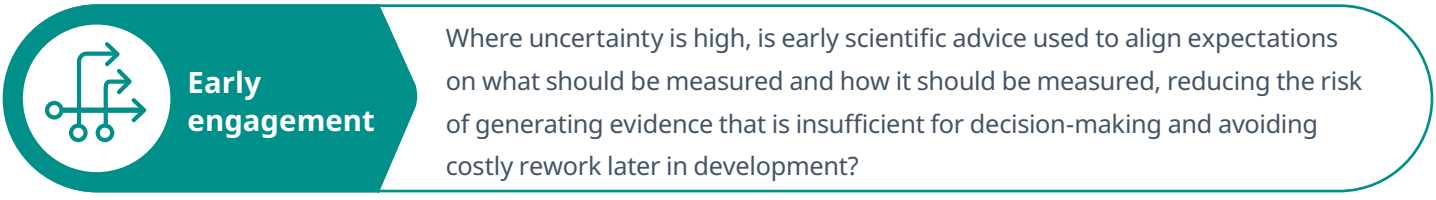












How IQVIA supports PED strategies

IQVIA's Patient Centered Solutions Strategic & Scientific Research consulting team supports end-to-end PED strategy, from early evidence planning and COA strategy through COA selection and validation, qualitative and preference research, and submission-ready evidence packages that support regulatory, HTA, and market access decision-making. Our work emphasizes fit-for-purpose design, methodological rigor aligned to decision intent, and evidence packaging that facilitates efficient review across regions. In practice, this includes COA strategy and dossier development (across COA types), qualitative, quantitative (including psychometric and efficacy-based analyses), and mixed-methods approaches (including longitudinal in-trial interview designs when appropriate), and patient preference research anchored to benefit–risk and endpoint decisions.

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About the author

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Barbara is an accomplished regulatory affairs leader with 30 years of pharmaceutical industry experience in regulatory strategy and writing. With expertise in leading global health authority interactions including scientific, pre-submission, and advisory committee meetings, Barbara is skilled in navigating complex regulatory frameworks, identifying and mitigating risks, and ensuring submission excellence and inspection readiness. She is a trusted cross-functional leader adept at aligning regulatory strategy with evolving health authority expectations and is passionate about patient-focused drug development. Prior to joining PCS, Barbara built her career in global regulatory strategy and regulatory writing in small, mid-sized, and large pharmaceutical organizations, specializing in cardiovascular, CNS, oncology, and rare disease therapies. She earned her BS in Neuroscience and MS in Health Informatics at the University of Pittsburgh. Barbara has Regulatory Affairs Certification for drugs and medical devices and is currently working towards certification in regulatory compliance.

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