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The professional society for health economics and outcomes research

Improving healthcare decisions

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January 30, 2026

Dear European Medicines Agency (EMA):

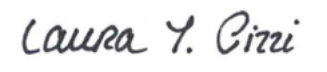
ISPOR – The Professional Society for Health Economics and Outcomes Research - is pleased to respond on behalf of its membership to your consultation entitled "**Patient experience data (PED) reflection paper**."

ISPOR is a scientific and educational society with many of its members engaged in evaluating health technologies, including pharmaceuticals, medical devices, and other interventions. We have a large membership based in 110 countries globally, across a range of disciplines, including health economics, epidemiology, public health, pharmaceutical administration, psychology, statistics, medicine, and more, from a variety of stakeholder perspectives, such as the life sciences industry, academia, research organizations, payers, patient groups, government, and health technology assessment bodies. The research and educational offerings presented at our conferences and in our journals are relevant to many of the issues and questions raised in this request for information.

The response to this consultation was led by the ISPOR Scientific and Health Policy Initiatives Team. Comments were solicited from the Health Science Policy Council, ISPOR Patient Council, Partnership Group, several ISPOR Special Interest Groups (Clinical Outcome Assessment, Health Preference Research, Patient-Centered), and the Community of Interest – Health Policy. The attached document provides a summary based on their comments. We hope they prove useful.

ISPOR would be happy to answer any questions about our response, serve as a partner, or participate in any follow-up consultations on the relevant program items mentioned in the report. We sincerely value our collaboration with EMA and strongly support the agency's efforts to advance patient-centered regulatory evidence.

Sincerely,



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European Medicines Agency - Patient experience data (PED) reflection paper

We welcome the European Medicines Agency's (EMA) release of the Patient Experience Data (PED) reflection paper, which highlights the importance of systematically conducting patient-centered research that directly reflects patients' lived experiences, without intermediary interpretation. ISPOR defines patient engagement in research as "the active, meaningful, and collaborative interaction between patients and researchers across all stages of the research process, where research decision making is guided by patients' contributions as partners, recognizing their specific experiences, values, and expertise,"[1] a definition that has been referenced by authorities such as CIOMS and could be helpful to reference in the final PED reflection paper.

Overall, the paper provides a timely signal that patient experience data should play a more explicit role in medicine development and marketing authorization applications (MAAs) for human medicines. By articulating the value of data that directly captures patients' lived experiences, this paper reinforces the importance of the patient voice in regulatory decision making. We find this reflection paper well written and believe it will encourage earlier and more meaningful engagement among medicine developers, patients, and regulators, ultimately supporting more patient-centered development and evaluation of medicines.

We support the paper's objective to advance more systematic consideration of patient-derived evidence across the medicine lifecycle. While the paper clearly articulates the importance of PED, it remains largely high-level and provides limited clarity on how PED are expected to inform regulatory decision making in practice.

Qualitative PED and patient preference studies (PSS) are uniquely suited to capture patient priorities, acceptable risk, treatment trade-offs, lived experience, and contextual meaning that cannot be inferred from quantitative outcomes alone. For many patients, PPS are the primary means by which values, priorities, and acceptable uncertainty can be expressed. Their decision-relevant role should therefore be more explicitly articulated to justify participant burden and developer investment.

The reflection paper would benefit from more practical, implementation-oriented strengthening to support consistent uptake and reduce uncertainty. This can be achieved by including:

- Illustrative examples of when and how PED is most relevant at different stages of development, including early and non-clinical stages
- Case studies demonstrating how PED has meaningfully informed regulatory decisions
- Reflections on instances where PED could have added value but was not incorporated
- Clear differentiation between formal qualitative research methodologies and informal patient engagement approaches, each of which has a value to PFDD

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but the former providing a higher level of scientific rigor
- Practical scenarios demonstrating the value of early patient experience data for developers (eg improved endpoint selection, trial design, or development efficiency)

The paper risks understating the maturity of established PED methodologies, particularly for PROs, PROMs, and PPS, where robust validation frameworks and good practice standards are already widely implemented globally. Explicit cross-referencing to existing guidances, such as the European Medicines Agency *Engagement Framework: EMA and patients, consumers, and their organisations*,[2] the FDA Patient-Focused Drug Development Guidance series,[3] the Council for International Organizations of Medical Sciences (CIOMS) *Patient involvement in the development, regulation and safe use of medicines* report,[4] the Innovative Medicines Initiative (IMI) PREFER Project,[5] and the European Medicines Agency *ICH E22 General considerations for patient preference studies – Scientific guideline,*[6] as well as established methodological recommendations, such as the ISPOR Reports on *Quantitative Benefit-Risk Assessment in Medical Product Decision Making: A Good Practices Report of an ISPOR Task Force*[7] and *A Roadmap for Increasing the Usefulness and Impact of Patient-Preference Studies in Decision Making in Health: A Good Practices Report of an ISPOR Task Force*[8]– would further enhance usability, promote consistency, and reduce uncertainty regarding expectations and evidentiary standards, as these documents provide widely recognized guidelines for decision-relevant patient-derived evidence.

Across PED types, additional clarity would also be beneficial regarding methodological rigor and representativeness. Representativeness should be framed as a contextual and multidimensional concept rather than a binary criterion, with transparent discussion of limitations when full representativeness is not feasible. Ethical considerations, such as participant burden, reasonable compensation, transparency regarding potential conflicts of interest, and feedback to patient participants, are essential for qualitative PED and PPS. Greater transparency regarding how PED is evaluated and how it influences regulatory outcomes would further strengthen trust and credibility.

It would also be significant to state that early scientific advisement to health technology developers regarding PED can be sought as a component of the Joint Scientific Consultation process which EMA has implemented under EU regulation. Doing so would demonstrate EMA's global regulatory leadership and commitment to engaging patients early in the development process, a need that other global regulators have fallen short of.

Finally, strengthened definitions of key concepts, such as qualitative PED and patient engagement, and simplification of technical phrasing where possible, would improve clarity and accessibility for a broad stakeholder audience. In this context, referencing established definitions of patient engagement in research, such as the ISPOR definition[1], can help clarify distinctions between patient engagement activities, qualitative PED, and other forms of patient-derived evidence, supporting consistent application across regulatory submissions.

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In summary, we support the Agency's leadership in advancing patient-centered approaches. Further clarification and strengthening the paper's focus on decision relevance, early and systematic integration, methodological clarity, transparency, and consistency, and clearer alignment with existing relevant guidance would further enhance its value for patients, developers, and regulators.

We welcome a more fulsome discussion of the comments ISPOR received, our current scientific initiatives related to patient-centered research, and how they might inform EMA's efforts. We also welcome discussion with EMA on gaps in the methods or stakeholder discussions that ISPOR can help to address.

We acknowledge the following ISPOR Special Interest Group members, Holly Peay, Angie Botto-van Bemden, Kate Williams, Elizabeth Manias, Marco Boeri, Sunil Shrestha, Jayeshkumar Kanani, Tasnim Suhail Abu Al Khair, Mohammad Ahmmad Mahmoud Al Zoubi, Yin Min Kyaw, Russ Montgomery, Alessandra Girardi, and Franklyn Opara, for their review and assistance in assembling these comments. We also acknowledge ISPOR staff Laura Pizzi, Clarissa Cooblall, Sahar Alam, and Kelly Lenahan for their collaborative effort in thoughtfully synthesizing the response to this important reflection paper.

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