ACRO Innovate Advocate Collaborate

29 January 2026

To: EMA Committee for Human Medicinal Products (CHMP) and Pharmacovigilance and Risk Assessment Committee (PRAC)
From: Karen Noonan, Senior Vice President, Global Regulatory Policy
RE: EMA Reflection paper on patient experience data
EMA/CHMP/PRAC/148869/2025 (18 September 2025)
Submitted electronically – via required EMA Excel form – on 29 January 2026

Line from* (line Nr or 0 for general comment)	Line to* (line Nr or 0 for general comment)	Section number	Comment and rationale (to go to next line within the same cell use Alt + Enter)	Proposed changes / recommendation (if applicable - to be used if you want to propose specific text changes)
0	0		Founded in 2001, the Association of Clinical Research Organizations (ACRO) is non-profit trade association representing the world's leading clinical research and technology organizations, which provide specialized services that are integral to the development of drugs, biologics and medical devices that enable patients to live longer, healthier, and more productive lives. ACRO members provide a wide range of specialized services across the entire spectrum of development - from preclinical, proof of concept, and first in human studies through post-approval, pharmacovigilance, and health data research. ACRO member companies employ nearly 400,000 people worldwide and conduct research in every global region.	

			ACRO supports the development of this reflection paper as incorporation of patient experience data throughout clinical development is critical to ensure the generation of meaningful clinical evidence from clinical trials. In particular, ACRO has supported the practical integration of patient experience data within clinical trials through its thought leadership on Decentralised Clinical Trials. In ACRO's White Paper on DCTs, ACRO experts cited patient-centricity as a key advantage of decentralized clinical trials, including "using patient-reported outcomes as study endpoints" and providing two case studies of electronic patient-reported outcomes (ePROs). [ref: ACRO DCT White Paper 2020]	
109	113	2.1.1	Given treatment preferences are listed as an example of PED, ACRO suggests also including quantitative methods used to collect treatment preferences in the examples of ways of collecting PED.	Replace text with: "PED can be collected using quantitative methods e.g., quantitative surveys exploring relevant clinical outcomes or minimum relevant thresholds for patients; quantitative surveys exploring treatment preferences (e.g. using a discrete choice experiment or other preference elicitation method); instruments for health-related quality of life [HRQoL] or other patient-reported outcome measures [PROMs]), qualitative methods (e.g., interviews, focus groups or qualitative surveys that reflect the wider perspective of patients' experience) or mixed methods that integrate both quantitative and qualitative approaches"
165	166	2.1.2.2 Table 1	Lines 97-99 imply that PED does not include data that has had input or interpretation from a healthcare professional. ACRO notes that Table 1 includes CROs (clinician-reported	

			outcomes) as an example of PED methods. ACRO would recommend clarification and alignment of the two sections of the Reflection Paper.	
165	166	2.1.2.2 Table 1	ACRO recommends adding examples of Observer reported outcomes (ObsROs) to the table, in order to illustrate the previous point (lines 97-99) of carers being a source of PED.	Additional row: “Observer Reported outcomes (ObsROs)”
165	166	2.1.2.2 Table 1	Lines 97-99 imply that PED does not include data that has input or interpretation from a device. ACRO notes that Table 1 includes “Digital/AI-base methods” as an example of PED methods. ACRO would recommend clarification and alignment of the two sections of the Reflection Paper.	
176	177	2.2 Table 2	It is unclear what is meant by "non-clinical research". For example, would this also include non-interventional research or market research? It is feasible that "non-clinical" research could also contribute to development and selection of appropriate endpoints/PROs.	Consider changing the first row header to "Non clinical-trial research," assuming the intent is to have that row serve for ALL non-trial research. Consider also adding examples.
176	177	2.2 Table 2	The example given under “Assessment of major contribution to patient care” is not clear.	Replace text with: "Establish whether a medicine represents a major contribution to patient care compared with relevant comparator treatments across various regulatory settings, (e.g., for conditional marketing authorization, or for granting of data exclusivity and market protection for orphan medicines)."
179		2.3	ACRO recommends including guidance on the relevance of PED collected from carers as proxy for patients and the	

			circumstances where this may be appropriate.	
199	200	2.3.1	ACRO notes that reference 23 from the FDA explicitly discourages proxy-reporting of outcome measures for certain groups. It would therefore be helpful to clarify in this section the difference between Observer reported outcomes (ObsRO) and proxy-reported outcomes.	
206	208	2.3.2	ACRO would recommend inclusion of specific terms to further explain validation of PROMS. For example, it would be helpful to include the terms “content validation” and “linguistic validation” to give examples of possible “appropriate techniques”.	
254	265	2.3.2.1	This section appears to merge qualitative patient preference studies (PPS) and qualitative research on patient experiences more generally, including experiences of the disease and its impact on daily life which are not inherently related to patient preferences. It is important to distinguish between qualitative patient experience studies and patient preference studies, as they serve different scientific and regulatory purposes. Qualitative patient experience studies focus on understanding the lived experience of the disease, its burden, its impact on functioning and daily life, and experiences with treatment. These studies generate foundational insights into symptoms, impacts, expectations,	

			treatment perceptions, and what patients consider meaningful. This type of research can be conducted either outside the clinical trial context (e.g. qualitative exploratory studies) or embedded within a trial (e.g. in-trial interviews).	
275	278	2.3.2.2	ACRO recommends including reference to sample size, as discrete choice experiments are not feasible with small sample sizes e.g. in rare diseases.	Replace text with: "While most quantitative PPS research has been conducted using discrete choice experiments, the selection of the most adequate method depends on multiple factors, such as the complexity of the method based on the study population, the feasible sample size (e.g. in rare diseases with <100 people methods such as thresholding are more appropriate to DCEs), and their capacity to answer the research question as well as their efficiency in doing so."
294	297	2.3.3	ACRO notes that EMA has "developed several tools for patient engagement that are applied at various points during EMA's regulatory processes to provide insights into how patients experience their condition, symptoms, burden of disease, burden of treatment, quality of life and treatment preferences." It would be helpful to provide references to any additional resources that are available.	
457	458	2.4.4	ACRO notes the line "Other sources of PED, less conventional and yet to be established, include mobile health technologies and social media data." This is followed by a generalized description of mobile health technologies (including wearables) and social media.	"Other sources of PED, which are being established, include mobile health technologies and social media data."

			Whilst ACRO acknowledges the relatively new use of these data sources, they may be useful as noted in lines 459-479 and this is an area of ongoing development. ACRO would therefore recommend rewording of this line to future-proof the reflection paper. ACRO would also recommend the addition of information in this section to give guidance on how developers and researchers should proceed in this area, for example, by seeking scientific advice on validation of the proposed PED.	
503	509	2.5.2	A caveat could be added noting that, in qualitative research, sample size is often driven by conceptual saturation which is the point at which no new meaningful information emerges from additional interviews. In the context of a clinical trial, the representativeness of the trial sample is also an important factor in determining sample size. However, it is generally not necessary to interview the entire trial population to address qualitative research questions. Instead, a selected estimated minimum subset of participants, based on the number of treatment arms (triple blinded), is typically sufficient to capture the range of relevant experiences and support robust qualitative insights.	
573	587	2.5.9	This section is titled “Perceived lack of value”. This section heading may cause confusion and send conflicting messages on EMAs view on the value of PED data.	Replace Title with: “2.5.9. Stakeholder alignment on PED value"

			ACRO would therefore recommend consideration of an alternative section title.	
			ACRO thanks the EMA for the opportunity to provide this comment. Please contact ACRO if we can answer any questions or provide additional information.	