EMA
EUROPEAN MEDICINES AGENCY

Emerging topics from PED reflection paper consultation

PCWP-HCPWP – 4 February 2026

Presented by Rosa Gonzalez-Quevedo
Public and Stakeholders Engagement Department

Progress overview

2022

EMA workshop on PED

2023

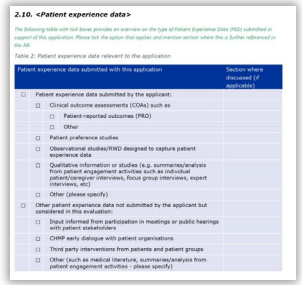
PED expert group and action plan

Creation of EMA PED community

2024

Drafting of PED reflection paper

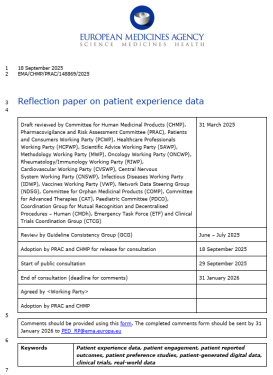
Update CHMP AR



2025

Publication of PED RP for public consultation

Survey on PED in therapeutic areas



2026

Publication of final PED RP

Survey analysis

Evaluation of updated CHMP AR

Background

- The reflection paper was drafted by a **multidisciplinary group of experts** from CHMP, PRAC, PCWP, HCPWP, SAWP, MWP, ONCWP, RIWP, CVSWP, CNSWP, IDWP, VWP, NDSG, COMP, CAT, PDCO, CMDh, ETF and CTCG
- It discusses types and sources of PED, general principles and elaborates on the use and value of PED across the medicine lifecycle
    - It is **complementary to ICH's work** on patient focused drug development guidelines
- Stakeholders are encouraged to embed **PED across all stages of medicine development**
    - This can be achieved by **liaising early with EMA through scientific advice/qualification of novel methodologies**
- It was published on 29 September 2025 for 4-month public consultation **ended on 31 January 2026**

Table of contents

A few numbers

- 112 stakeholders commented
- 2117 rows of comments received
- Comments received on all parts of the document without exception

Geography	
EU	60
Europe non-EU	22
Global	20
US	5
Asia	2
South America	1
Unknown	2
TOTAL	112

Stakeholder types		
PCO	39	Incl. 14 eligible PCOs
Industry	37	
Multistakeholder consortium	10	
Research / Academia	7	
HCPO	4	Incl. 3 eligible HCPOs
public health body	1	
Other	9	
Unknown	5	
TOTAL	112	

General comments

General	202

"The reflection paper is welcomed as a **valuable consolidation of current thinking**, definitions, and practices related to patient experience data (PED). It provides a helpful shared reference point across stakeholders and **acknowledges the growing importance** of patient experiences across the medicines development lifecycle."

"The Reflection Paper is **timely and highly relevant**. The increasing use of patient experience data (PED) across clinical development, regulatory assessment, and post-authorisation evidence generation creates a **clear need for shared principles and expectations**. The document represents an important step towards greater consistency and transparency in how PED are considered within the EU regulatory framework…. At the same time, the Reflection Paper remains largely descriptive and principle-based and does **not yet provide sufficient practical guidance** on how the use of PED could be meaningfully strengthened in practice."

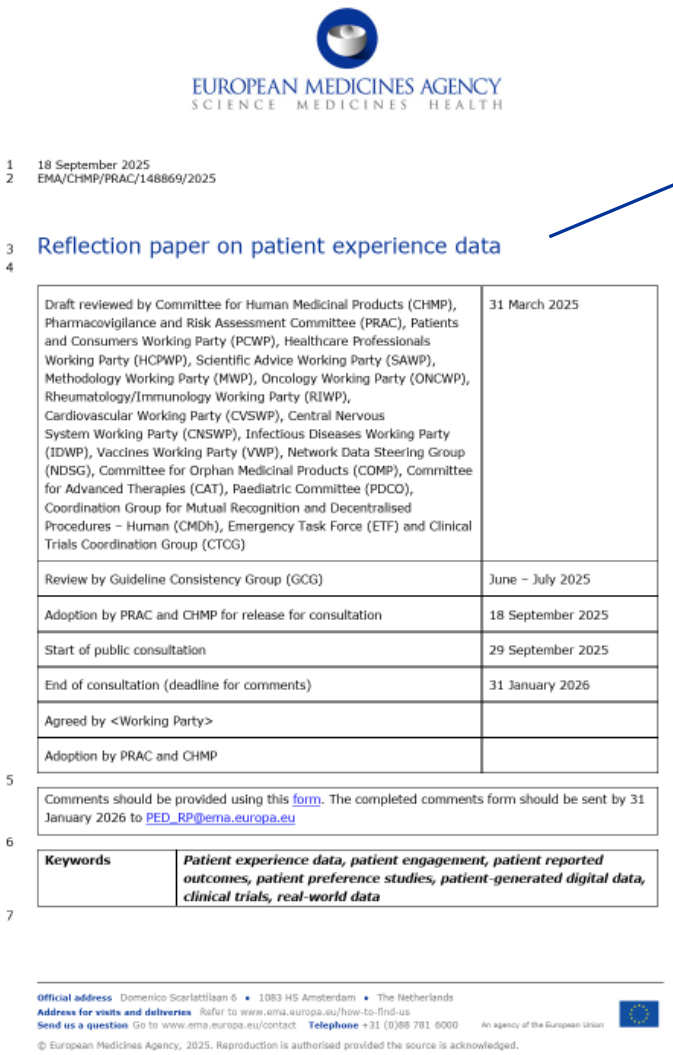
"While we understand the intention to remain non-prescriptive, **additional methodological direction would greatly support consistency and quality**, particularly given the diversity of PED methods"

"The language is predominantly encouraging ("should be considered") rather than establishing minimum expectations. For meaningful change, the **paper must evolve from describing PED to prescribing** how to implement it systematically across all development phases."

"We greatly appreciate the efforts that went into the development of this reflection paper to outline the EU regulatory approach to PED…. However, the reflection paper highlights a **clear need for greater international harmonization** of PED terminology, methods, and standards to facilitate global regulatory alignment and drive patient-centric drug development."

"Qualitative and mixed-methods approaches are mentioned in the reflection paper but their **role remains insufficiently specified**."

Title and keywords



EUROPEAN MEDICINES AGENCY
SCIENCE MEDICINES HEALTH

18 September 2025
EMA/CHMP/PRAC/148869/2025

Reflection paper on patient experience data

Draft reviewed by Committee for Human Medicinal Products (CHMP), Pharmacovigilance and Risk Assessment Committee (PRAC), Patients and Consumers Working Party (PCWP), Healthcare Professionals Working Party (HCPWP), Scientific Advice Working Party (SAWP), Methodology Working Party (MWP), Oncology Working Party (ONCWP), Rheumatology/Immunology Working Party (RIWP), Cardiovascular Working Party (CVSWP), Central Nervous System Working Party (CNSWP), Infectious Diseases Working Party (IDWP), Vaccines Working Party (VWP), Network Data Steering Group (NDSG), Committee for Orphan Medicinal Products (COMP), Committee for Advanced Therapies (CAT), Paediatric Committee (PDCO), Coordination Group for Mutual Recognition and Decentralised Procedures – Human (CMDh), Emergency Task Force (ETF) and Clinical Trials Coordination Group (CTCG)	31 March 2025
Review by Guideline Consistency Group (GCG)	June – July 2025
Adoption by PRAC and CHMP for release for consultation	18 September 2025
Start of public consultation	29 September 2025
End of consultation (deadline for comments)	31 January 2026
Agreed by <Working Party>	
Adoption by PRAC and CHMP	

Comments should be provided using this form. The completed comments form should be sent by 31 January 2026 to PED_RP@ema.europa.eu

Keywords	***Patient experience data, patient engagement, patient reported outcomes, patient preference studies, patient-generated digital data, clinical trials, real-world data***

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Title: 2 comments received

- Propose to **change the overarching concept** from “Patient experience data (PED)” to “Patient-reported data (PRD)” or “Patient-generated data (PGD)” throughout the document.
- Consider **adding ‘for regulatory use’** to read: Reflection paper on patient experience data for regulatory use”.

Keywords:

- Remove “patient-generated digital data”
- Consider **adding “rare diseases”** OR “rare and chronic diseases”

1. Introduction

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"The introduction doesn't clearly establish **why EMA needs PED"**

"The scope appropriately recognises the value of patient experience data but would benefit from a clearer emphasis on contexts where patient experience is particularly critical, such as **paediatric populations**, **rare diseases** and **severe or life-limiting conditions**."

"Although the scope of the paper is not to provide detailed methodological guidance, the **continued absence of internationally harmonised guidelines remains a significant barrier** for sponsors."

"As academic researchers also conduct research in various stages during the lifecycle of drugs, we suggest that **academia** is explicitly mentioned."

"Explicitly acknowledging the **need for standardised approaches"**

"PED should also apply to **combination products** (drug–device) and **medical devices**, not just medicines"

"…It would be beneficial to acknowledge that PED may also have **value in non-medical or cross-sectoral research contexts"**

"Developers **lack clarity on acceptable standards"**

2.1. The EU regulatory approach to PED

"The requirement that PED must not be **subject to third-party interpretation** is not realistic. PROs are scored, surveys analysed, registries aggregated, and preference studies modelled — **all require scientific processing**."

"The paper does not explicitly refer to **Patient Reported Experience Measures** (PREMs) as a distinct and relevant category of patient experience data"

"PED should also include the **psychological and economic burden on patients** and caregivers"

"Patient Engagement activities represent an important source of PED and should also be considered as a way to contextualize data generated through other methods"

"International harmonisation on what data constitutes as PED and its value is paramount in ensuring that the relevant data is collected, analysed, interpreted, and reported appropriately"

"While the principle that PED should not be "subject to third-party interpretation" is understandable, its application in **pediatric contexts**, particularly with very young children, may be problematic"

2.2. Use and value of PED along the medicine's lifecycle

"Table 2 is very useful but it is quite dense. Consider **adding a short synthesis paragraph** highlighting a few high-impact moments where PED tends to be most underused today"

Extend Table 2 with an additional column, labeled "**Regulatory touchpoints/deliverables**".

"Consider clarifying whether the table is intended to be **illustrative rather than exhaustive** would improve clarity."

"Table 2 is helpful but broad. Healthcare stakeholders will want **examples"**

"In the section of the table for "Clinical trial design" - **Refine study design and objectives"**

"Table 2 does not include examples related to participant burden or the use of patient input to assess and **mitigate burden** associated with study procedures"

"An additional important role for PED in clinical trial design is assessing clinically meaningful changes in **endpoints**, especially of novel endpoints for which there is little experience."

"Table 2 is helpful but key areas are **not elaborated enough** to be actionable"

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"When assessing patient-reported outcomes, it is important to account for **potential confounders**, such as co-medication, which may influence perceived treatment effectiveness."

"PROs aren't "normally" collected through **proxy-reported outcomes**. This is widely discouraged and not standard."

"Much **more detailed guidance** on validation and other regulatory requirements **is needed**."

"The paper could also acknowledge the **limitations of generic patient-reported outcome measures**, which are used across several disease areas…"

"Several **notable use cases for PPS have been omitted** from the examples."

"The terms **"PRO" and "PROM" are used inconsistently** throughout the paper."

"The document **does not address the validation** of PRO instruments."

"Given the very large number of PRO available, the reflection paper provides **too limited guidance on regulatory expectations** regarding PROs quality and validation."

"**Consider adding examples** or references where PPS has influenced regulatory decision making."

"We recommend **clarifying how and under what circumstances** PED, including PPS, will be considered in regulatory assessments."

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“Further clarification and guidance is needed on what it means to "meet quality standards equivalent to trial-based PED" for primary data collection outside of a clinical trial for regulatory assessment.”

“While real-world data and digital health technologies are recognized as potential sources of PED, the document does not specify the **standards or validation requirements** for their use in regulatory decision-making”

“The paper mentions "special populations such as older, frail or paediatric patients" only in passing. **Pediatrics requires dedicated subsection** addressing unique challenges”

“It would be helpful for the **section on mobile health technologies** to acknowledge that standards for assessing and validating these technologies already exist and are being applied by other regulators, such as the FDA”

“The document identifies various PED sources but does not address the potential for **OPEN DATA repositories**.”

“The current wording presents a reductionist view that does not reflect recent advances in **social media listening** (SML).”

“In order to reflect current developments in clinical research and regulatory science, it would also be appropriate to consider the inclusion of tools/health technologies employing **artificial intelligence** where relevant.”

2.5. Considerations for systematic implementation of PED

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"EMA could strengthen the reflection paper by articulating minimum **expectations for representativeness** planning and **transparent reporting** in PED submissions"

"**Global alignment** on patient experience data is critical for efficiency and consistency"

"The use of **AI must be discussed** in more detail, considering the collection, analysis and interpretation of PED using AI tools. The paper should have a dedicated section on this clearly providing recommendations about prohibited and allowed uses of AI."

"Transparency is mentioned, but the **internal review process** is not described."

"The reflection paper commits to transparency but would benefit from **standardised reporting formats** so applicants can present PED in a clear, comparable way across products and procedures."

"Publication of **successful case studies** on collecting PED and using them in regulatory processes would be useful both for patient advocacy groups Patient Advocacy Groups (PAG) and industry."

3. Conclusions

3. Conclusions 23

"Consider adding an explicit final statement that high-quality PED is enabled by **early planning, methodological rigor, and cross-stakeholder collaboration** on tools and standards, consistent with the barriers described earlier."

"Stakeholders are encouraged to embed PED across all stages of medicine development. This appears as a conclusion but is **not emphasised enough**."

"Consider **reaffirming** that PED and patient preference studies are **essential** to patient-centered regulation, rather than supplementary"

"We suggest highlighting the **relevance** PED can also have **for HTA and clinical decision-making**."

"Include a **timeline** or next steps"

"Important to acknowledge in the conclusions the **broad role** PED can have in regulatory & HTA assessments, as well as in clinical decision-making"

"Conclusion reiterates value but lacks concrete **next steps"**

References and glossary

"The reference list is **comprehensive and highly relevant**; however, there are several issues relating to inconsistent formatting, **broken or incomplete links**, missing or unclear citation details, and references that are not clearly linked to statements in the text."

"Lack of references to **relevant EMA guidelines** and prior reflection papers for context and completeness."

"The **definitions of PED types** in the glossary could be strengthened."

We recommend the inclusion of a glossary of all **PED-related terms**

list and define all the relevant **abbreviations** and definitions

Glossary should include **PED itself and types of PED**.

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Thank you

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