EMA
EUROPEAN MEDICINES AGENCY

Update on EMA's work on Patient Experience Data (PED)

MSP– 29 October 2025

Presented by Rosa Gonzalez-Quevedo, PhD
Public and Stakeholders Engagement Department

In this presentation

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Relevance of PED

- Patient Experience Data (PED) is data reflecting patients' experience **without input or interpretation by others** (PROs, patient preferences, data from patient engagement activities)

- **Patients' views or preferences** on medicines or living with a condition is particularly important for many medicines, such as cancer medicines, where quality of life may matter most to patients than more established endpoints (e.g. overall survival)

- Collection of PED using reliable and validated methodologies **can contribute to benefit/risk evaluation** to complement primary or secondary endpoints

- In particular, PROs can contribute to decision-making in **cases when "harder endpoints" have not reached maturity** by the cut-off point

- In the post authorisation phase, PED can be collected **as part of RWD (e.g. in registries) to generate supportive evidence**

EMA's Action Plan on PED – Priorities

Overall EU strategy and approach	Regulatory guidance with stakeholder input	Improve alignment, data quality and methodologies	Increase transparency	RWE and digitalisation	Training and resources
• Agree overall approach on PED with the Network	• **Reflection paper & Stakeholder consultation**	• Support ICH guidelines	• Inventory of PED use cases – scientific publications	• Involvement of PCOs in Big Data	• Collaborating experts
• List of priorities	• PCOs/HCPs - populating EMA data catalogues	• Mapping EU and international initiatives	• **Update of CHMP AR template**	• Proof of concept studies	• EU Network training centre
• Monitor implementation	• PCOs/HCPs -Data Quality Framework	• Support HTA/payer contribution to reflection paper	• Exploring update of medicine overview	• Literature review of use of PED in non-interventional studies	• Coordinate stakeholder requests
• **Network expert group**	• **Therapeutic area priorities**	• Workshops on qualification, registries	• Exploring update of OMAR template	• PED data sources in data catalogue	• Overview of projects on PED with EMA involvement
		• Ongoing projects	• Link to AI groups	• Learnings from ongoing SMA study	

EU regulatory approach to PED

PED should be systematically considered for informing medicines development

PED can be a **relevant contributor to the totality of evidence** throughout the medicine lifecycle

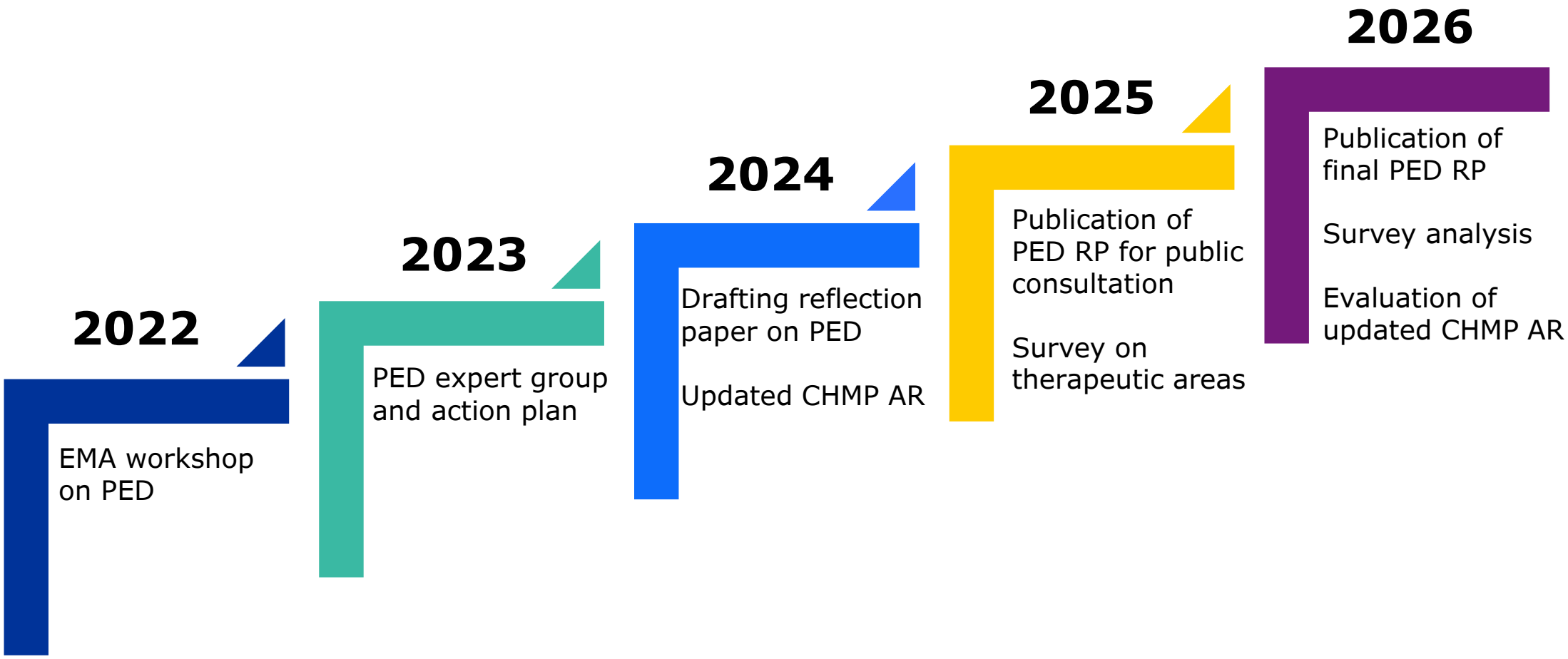
PED are **applicable to all stages of the medicine** from the earliest ones (including non-clinical stages) through to post-marketing

For PED to inform or **support regulatory benefit-risk assessment** and decisions the data should be of **high quality**

The resulting evidence should be generated using **robust and validated methodologies**

Measures that reflect patients' priorities should be included, where possible

Progress overview



PED reflection paper drafting group

Multidisciplinary drafting group set up with experts from the **EU Network**

Covering expertise within each Committee/working party

Committee/experts	Examples of areas covered
CHMP	All aspects of benefit-risk assessment
PRAC	ADR reporting, preference for risk minimisation activities
COMP	Rare disease/orphan medicines/ major contribution to patient care for significant benefit
PDCO	Paediatric aspects (e.g formulation)
CAT	Advanced therapies aspects
Working parties: SAWP, MWP, ONCWP, RIWP, CVSWP, CNSWP, IDWP, VWP Patients, consumers and HCP representatives ETF, NDSG, CTCG	Specific aspects to each group

Scope and key aspects of reflection paper

The reflection paper has been published on 29 September 2025:

- A path to better include patients' perspectives in the regulation of medicines | European Medicines Agency (EMA)
- Patient experience data (PED) reflection paper | European Medicines Agency (EMA)

Public consultation is open until **31 January 2026**

The RP is a **framework for discussion** or clarification particularly in areas where scientific knowledge is fast evolving or regulatory experience is limited

It describes **general principles** – it is not a methodological guidance.

It is **complementary to ICH** guidance work

It encourages **systematic consideration of PED** in medicine development programmes and regulatory submissions.

Target audience:

- medicine developers,
- regulators,
- Researchers, and
- patient groups who generate, collect and review PED

Elements of the reflection paper

- Introduction, Problem Statement and Scope
- The EU regulatory approach to PED
  - Scientific advice and qualification, Innovation Task Force, academia support
- Use and value of PED along the medicine's lifecycle
- Types of PED:
  - Clinical trials (PROs, PPS), patient engagement
- Sources of PED:
  - real-world data, safety surveillance systems, other potential sources
- Considerations for systematic implementation

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Scientific advice and qualification of novel methodologies

The EU approach is to encourage companies to liaise early with regulators during scientific advice or qualification to discuss best way to generate and collect PED and have a case-by-case discussion on their specific development plans

Scientific Advice

- Developer presents plans to develop a medicine and identifies questions and possible solutions
- EMA gives advice on developer's proposals
- Scientific advice can be provided on any PED scientific question (e.g. collection PED in clinical trials)

Qualification of novel methodologies

- Opinion on the acceptability of a specific use of a PED collection method, such as use of a novel PRO
- Advice on protocols and methods intended to develop a novel method with the aim of moving towards qualification

Use and value of PED along the medicine's lifecycle

The patient's voice is critical to better informing all stages of a medicine's development, from early development through regulatory assessment to post-marketing activities

Table 2: Examples of use and potential value of PED in the different stages of the lifecycle of a medicine

Research & development	
Non-clinical research	• Contribute to ensuring that non-clinical research questions address patients' unmet needs; • Help establish the preferred route of administration; • Identify existing products that can be optimised or extended to other indications and populations.[iii]

Clinical trial design	• Formulate trial questions that are most relevant to patients;
	• Refine study design and objectives by:
	  ○ selecting appropriate endpoints, including PRO instruments that reflect how patients feel and function;
	  ○ sharing knowledge on the natural course of the disease and standard of care (this could aid in the selection of the control group, if applicable, and target population);
	  ○ defining entry criteria to ensure that the most appropriate population is enrolled;
	  ○ supporting balanced gender participation and a gender-responsive approach that considers different treatment responses for men and women;
	  ○ defining preference and acceptability for comparators (placebo/standard of care) and dose;
	  ○ considering feasibility, relevance and specific aspects of studies for special populations (e.g., children, older and frail people);
	  ○ including QoL and ethical considerations;
	  ○ collecting input on informed consent and assent/agreement form and other documentation;
	• Increase willingness to participate in a trial, manage expectations and reduce the risk of dropouts from trials, thereby increasing the quality of the data.

Patient Reported Outcomes (PROs)

- Health outcomes that **directly report the patient's experience** of their health status without amendment or interpretation by a clinician or other party
- PROs can **enrich regulators' understanding** of a patient's experience related to symptoms, adverse effects and overall satisfaction, thus contributing additional evidence to support a medicine's approval
- Moreover, PROs can **strengthen the product labelling** by demonstrating improvement in daily functioning
- In the post-authorisation phase, PROs collected in registries and other real-world data sources can **help monitor the safety of a medicine**
- PROs are normally collected through patient-reported outcome measures (PROMs) or proxy-reported outcomes, such as questionnaires and surveys
- Need to ensure they are **standardised** and valid
    - apply psychometric principles,
    - methodological validation concepts and
    - appropriate techniques for questionnaire development and translation

Patient preference studies (PPS)

- Patient preference studies (PPS) can **complement evidence** from pivotal clinical trials to support decision making
- PPS include any **qualitative or quantitative assessment of the relative desirability or acceptability to patients** of aspects that differ among alternative health interventions (e.g. characterising medical need, selecting endpoints and estimating meaningful effect size, as well as identifying subgroups with different preferences)
- PPS may be **carried out by various stakeholders**: regulators, developers, patient groups, learned societies/clinicians or any other relevant ones
- PPS have not been extensively used in regulatory decision-making to date
- EMA considers it valuable to encourage the conduct of well-designed and reliable PPS and the use of PPS data
- '***Qualification of the IMI PREFER framework***' adopted by the CHMP in 2022 - provide a reference for a case-by-case approach to planning and conducting PPS
- PPS is also discussed at **ICH level** (ongoing drafting of ICH E22)

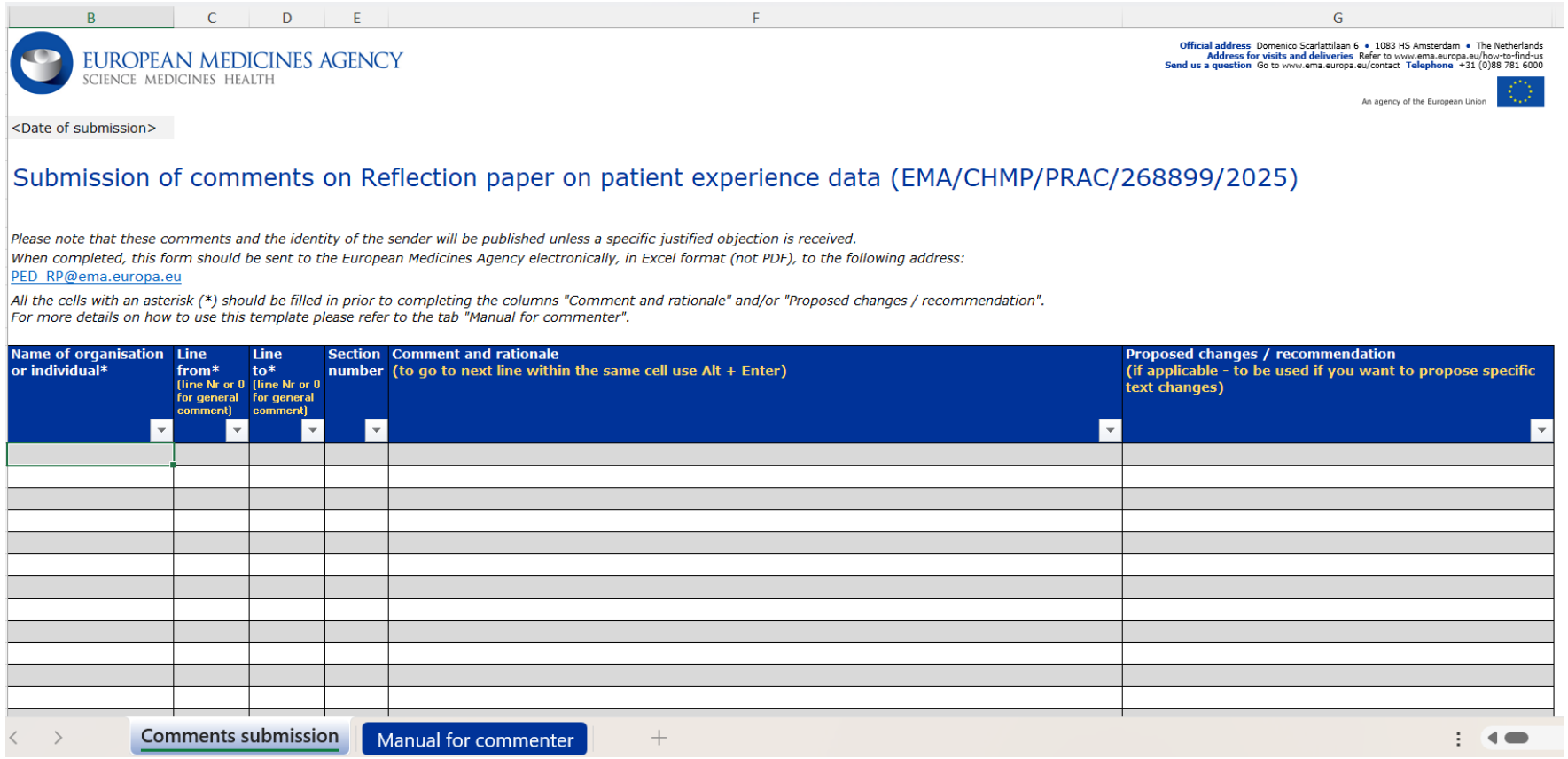
Data from patient engagement activities

- Although PPS or PROs are more established ways to collect PED during medicines development, data obtained through patient engagement activities should also be considered as an **important contributor to the totality of evidence**
- Patient engagement: **interactions with patients to gather their experience** with a disease and their preferences regarding treatments and outcomes
- A **variety of methodologies** can be used by medicine developers, regulators and other stakeholders to seek patients' input
- **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
- PED collected through EMA patient engagement activities are included in the assessment and **reflected in the assessment report**, alongside any PED that may be submitted as part of a marketing authorisation application

Considerations for implementation of PED

- ✓ Data quality
- ✓ Representativeness
- ✓ Study design
- ✓ Data collection methods & tools
- ✓ Challenges related to the use of PROs
- ✓ Participant burden
- ✓ Training and capacity building
- ✓ Language
- ✓ Perceived lack of value
- ✓ Transparency on the use of PED in regulatory assessment
- ✓ Global alignment on PED

Template to comment on the Reflection paper



EUROPEAN MEDICINES AGENCY
SCIENCE MEDICINES HEALTH

Official address Domenico Scarlattilaan 6 ● 1083 HS Amsterdam ● The Netherlands
Address for visits and deliveries Refer to www.ema.europa.eu/how-to-find-us
Send us a question Go to www.ema.europa.eu/contact Telephone +31 (0)88 781 6000
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<Date of submission>

Submission of comments on Reflection paper on patient experience data (EMA/CHMP/PRAC/268899/2025)

Please note that these comments and the identity of the sender will be published unless a specific justified objection is received.
When completed, this form should be sent to the European Medicines Agency electronically, in Excel format (not PDF), to the following address:
PED_RP@ema.europa.eu
All the cells with an asterisk (*) should be filled in prior to completing the columns "Comment and rationale" and/or "Proposed changes / recommendation".
For more details on how to use this template please refer to the tab "Manual for commenter".

Name of organisation or individual*	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)

Comments submission | Manual for commenter

https://www.ema.europa.eu/en/documents/template-form/submission-comments-reflection-paper-patient-experience-data-ema-chmp-prac-268899-2025_en.xlsx

The template for submitting comments during the public consultation is provided in **Excel**.

It is user-friendly, features expandable cells for ease of input and it includes a user manual.

Reflection paper timelines

CHMP & PRAC adoption of draft
Sep 2025

Launch public consultation
29 September 2025

End public consultation
31 January 2026

Implementation comments
February-March 2026

CHMP & PRAC adoption of final text
Q2 2026

Publication of reflection paper
Q2 2026

Conclusions

- The EU Network is progressing several initiatives on PED
  - ✓ A reflection paper has been published for a 4-month public consultation closing on **31 January 2026**
- The reflection paper discusses types and sources of PED, general principles and elaborates on the use and value of PED across the medicine lifecycle
  - ✓ In addition to well-established ways to collect PED (e.g. PROs, PPS) data obtained through patient engagement activities are also an important contributor to the totality of evidence
- It is complementary to ICH work on patient focused drug development guidelines
- PED can inform medicine development and regulatory submissions, by providing patient insights that can be valuable for the assessment of marketing authorisation applications, as well as in the post-marketing setting
- Stakeholders are therefore 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, to enable case-by-case discussions on specific development plans and regulatory submissions

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Thank you

PED_RP@ema.europa.eu

https://www.ema.europa.eu/en/patient-experience-data-ped-reflection-paper

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