EMA
EUROPEAN MEDICINES AGENCY

Patient Experience Data in development programs and regulatory decision-making

ISG meeting on 30 June 2025

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In this presentation

- Introduction: EMA initiative on patient experience data
- Update on key deliverables:
  - EMA reflection paper
  - Transparency
- Conclusions

What is patient experience data (PED)

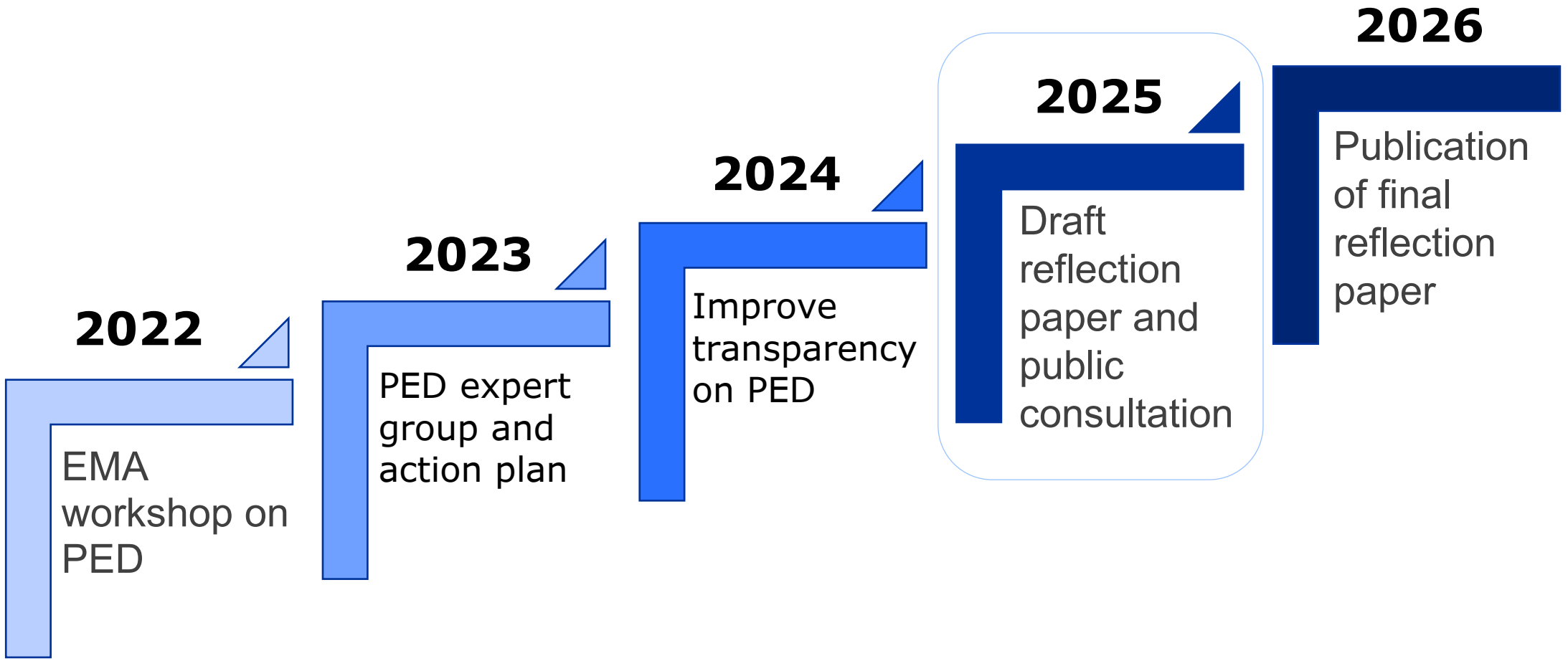
Current working definition:

- PED is data reflecting patients' experience **without input or interpretation** by others
- This can include, but is not limited to, health/functional status, symptoms, disease course, treatment preferences, quality of life, side effects…
- PED can be **generated, collected and submitted by different stakeholders** – as long as the data are confirmed to **directly reflect** patient experience
- PED can be **quantitative or qualitative** and can be collected in clinical trials or real-world settings
    - Includes e.g. patient-reported outcomes (PROs), patient preference studies (PPS), data from patient engagement activities

Patient experience data in the EU

- Reinforcing patient relevance in evidence generation is a key priority in the Regulatory Science Strategy and the European medicines agencies network strategy (See EMANS to 2025 and EMANS to 2028)
- Collection of PED using reliable and validated methodologies can contribute to benefit/risk evaluation to complement primary or secondary endpoints
- PED also relevant for implementation of the EU HTA regulation in value assessments that inform subsequent decisions by payers
- Post-approval PED can be collected as part of RWD (e.g. registries, patient reports) to generate evidence
- Progress in recent years on inclusion of PED in medicines development and regulation – but further work needed especially on methods, data quality

Progress in PED



Reflection paper on EU approach to PED

- **Key action** derived from the 2022 PED workshop – requested by stakeholders
- **Framework for discussion** or clarification particularly in areas where scientific knowledge is fast evolving or regulatory experience is limited
- **General EU framework or principles** – not methodological guidance – complementary to ICH guidance work
- Draft currently undergoing internal consultation and review
- **Public consultation** foreseen in late 2025

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 and Sources of PED – clinical trials (PROs, PPS), patient engagement, real-world data, safety surveillance systems, other potential sources
- Considerations for systematic implementation

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

Transparency on PED

- **Key action** from 2022 workshop
- As part of wider revamp in 2024 the CHMP assessment report template has been revised to capture different types of PED in more structured way
- New template implemented in Q1 2025 – available on EMA's website and direct link

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[insert only for CHMP adopted doc & add EMA header and footer]
Amsterdam, <insert full date>
<insert Doc. Ref.>
<Committee for Medicinal Products for Human Use (CHMP)>

<CAT/>CHMP assessment report for <initial marketing application><EUM4All (Art 58) scientific opinion>

Rapporteurs' Day<60*><82>assessment report - Overview and list of questions> *or* <DRAFT> <CAT><CHMP> Day <90*><120> list of questions *or* <DRAFT> <CHMP><CAT> Day <120*> <180> list of outstanding issues *or* Final <CAT/>CHMP assessment report
**in case of accelerated assessment*

EMA to ensure the correct text is reflected above at each milestone

<Product Name>

<International non-proprietary name> or <Common name**>:
*[**e.g. for vaccines and some ATMPs]*

Procedure No.

Transparency on PED – Dedicated PED section

2.10. <Patient experience data>

The following table with tick boxes provides an overview on the type of Patient Experience Data (PED) submitted in support of this application. Please tick the option that applies and mention section where this is further referenced in the AR:

Table 2: Patient experience data relevant to the application

Patient experience data submitted with this application	Section where discussed (if applicable)
☐ Patient experience data submitted by the applicant:	
☐ Clinical outcome assessments (COAs) such as	
☐ Patient-reported outcomes (PRO)	
☐ Other	
☐ Patient preference studies	
☐ Observational studies/RWD designed to capture patient experience data	
☐ Qualitative information or studies (e.g. summaries/analysis from patient engagement activities such as individual patient/caregiver interviews, focus group interviews, expert interviews, etc)	
☐ Other (please specify)	
☐ Other patient experience data not submitted by the applicant but considered in this evaluation:	
☐ Input informed from participation in meetings or public hearings with patient stakeholders	
☐ CHMP early dialogue with patient organisations	
☐ Third party interventions from patients and patient groups	
☐ Other (such as medical literature, summaries/analysis from patient engagement activities - please specify)	

6.3.8. <Patient experience data (PED)>

FACTUAL. This section is to be completed by the Rapporteur. Co-Rapporteur only to add if in disagreement or major omission.

Patient experience data (PED) are data collected through a variety of methodologies, including patient engagement activities, that directly reflect the experience of a patient or caregiver without interpretation by a healthcare professional, other third party, or (AI-based) device.

If patient experience data were submitted, provide a summary of such data. This may include PED from quantitative sources (e.g., patient reported outcome or experience measures, patient preference surveys), as well as PED from qualitative sources (any information obtained as part of patient engagement activities that reflect the wider perspective of patients' experience, e.g., outcomes of focus groups or interviews).

Describe whether the data come directly from the patients or caregivers, or if it was collected and submitted by other parties (advocacy group, researcher, developer, etc.).

If PED were submitted by the applicant, please describe their intended purpose (e.g., specify whether the data were collected to gather insights on an exploratory trial outcome, to inform the benefit-risk assessment, to enhance understanding of patient quality of life, or for other specific uses). In cases where there was CHMP early dialogue with patient organisations, please summarise the information received.

<Text>

In cases where there was CHMP early dialogue with patient organisations, please summarise the information received.

<Text>

Conclusions

- ✓ **EU regulators welcome PED** as important contribution to the totality of evidence
- ✓ EMA is working collaboratively to enable its broader use in regulatory decision-making
- ✓ **Key deliverables of EMA initiative on PED:**
  - EMA **reflection paper** will outline a European regulatory approach to PED:
    - Scientific advice + qualification of novel methodologies
    - Methodological work and guidance / harmonisation undertaken via ICH
    - Draft RP to be published in **late 2025** for **3-month public consultation**
  - **Increased transparency** on use of PED:
    - New template for CHMP assessment report (implemented)
- ✓ Currently **relatively low proportion are included in SmPC → future area for reflection**

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Thank you

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