Patient experience data - Reflection paper and upcoming consultation

PCWP/HCPWP and all eligible organisations meeting
20 November 2024

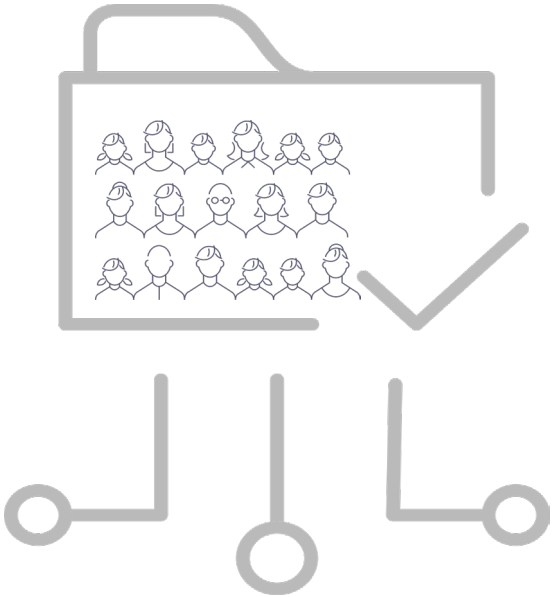
Presented by Rosa Gonzalez-Quevedo, PhD
Public and Stakeholders Engagement Department, European Medicines Agency

An agency of the European Union

Outline

- Patient experience data in the EU
- Update on progress on PED
- EMA reflection paper on PED
- Transparency
- Conclusions

Patient experience data (PED) in the EU



- **Data reported directly by patients or their carers** without interpretation by clinicians or third parties
- **Reflects patients' experience of their health conditions** and preferences on medicines
- **Proposal for an EU definition** as part of the EMA 2022 workshop
  - Definition to be agreed with stakeholders
- **Types of PED**:
  - Patient-reported outcomes (PRO), patient preference studies (PPS), data from patient engagement
  - Quantitative and qualitative data, clinical trials or RWD contexts

Patient experience data in the EU

- Reinforcing patient relevance in evidence generation is a key priority in EMANs and the Regulatory Science Strategy
- Patients' views on medicines or their condition are particularly important when quality of life can matter as much or more to patients than established endpoints (e.g. overall survival)
- Collection of PED using reliable and validated methodologies can contribute to benefit/risk evaluation to complement and support 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 phase PED can be collected as part of RWD (e.g. registries, patient reports) to generate evidence

Opportunities to improve

- Although there has been progress in the EU in recent years, PED are still not systematically included in all aspects of medicines development and regulation
- Stakeholder calls for progress and guidance from EMA to ensure the EU does not stay behind
- To optimise the use of PED, more work is needed especially on:
    - Data collection methods
    - Data quality and completeness
    - Methodologies applied to PED

Update on progress in PED

√ 2022

EMA workshop on PED

√ 2023

PED expert group & Action Plan

√ 2024

Improve transparency on PED

2024-25

Reflection Paper & Public Consultation

Reflection paper on EU approach to PED

- **Reflection paper:** framework for discussion or clarification particularly in areas where scientific knowledge is fast evolving or regulatory experience is limited
- **Key action** derived from the 2022 PED workshop - requested by stakeholders
- **General EU framework or principles** – not a methodological guidance – complementary to ICH guidance work
- **Public consultation** foreseen in 2025

Reflection paper on PED – elements to be covered

1. Problem statement and scope
2. EU approach to PED
    - Definition of PED
    - EMA scientific advice and qualification of novel methodologies
3. Use and value of PED along the medicine lifecycle
4. Sources of PED (clinical trial and real-world settings)
5. Methods to collect PED (PROs and other clinical outcome measures, PPS, patient engagement)
6. Factors affecting implementation (e.g. legal, methodological, data quality and access, choice, acceptance/trust, global development)
7. Conclusion

Scientific advice & 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

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

Qualification of novel methodologies

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

Sources of PED

PED can be collected from a variety of sources:

- **Clinical trial settings**, e.g. PROs, patient preference data
  - Use of tailored, pre-planned research protocols for primary collection of PED in specific context of given trial is encouraged
- **Real-world settings**
  - Primary data collection – non-interventional studies – can support planned clinical trials e.g. by informing selection of patient-relevant endpoints
  - Secondary use of data – e.g. registries, EHRs, administrative databases – can together with other health data enhance information for individual patient management, research etc. – increasing efforts to integrate PED within secondary data sources
  - Patient reports of ADRs – pharmacovigilance
  - Not yet fully established: mHealth / wearables, digital devices, social networks

Methods to collect PED: patient-reported outcomes

- PROs are **health outcomes reported directly by the patient** about their health status without amendment or interpretation by a clinician or anyone else
- PROs capture **patient-relevant** disease- or treatment outcomes, e.g. symptoms, functioning, or general multidimensional concepts such as health-related quality of life (HRQoL) – captured through generic or specific measures
- PROs can **complement clinical outcome measures** – enhancing regulators' understanding of patients' experience, e.g. regarding symptoms, adverse effects → more accurate evaluation of benefits and risks
- **Challenges** include selection and validation of instruments, missing data, potential bias or uncertain clinical relevance
- Stakeholders developing PROs for use in regulatory decision-making can ask for **CHMP qualification opinion** or **parallel scientific advice** (HTA/EMA) or support from EMA's **Innovation Task Force**

Methods to collect PED: patient preference studies

- PPS not yet extensively used in regulatory decision-making – complex, time and resource intensive with few standardized methods – but increasing interest in their use in medicines development
  - ➢ Qualitative (e.g. interviews, focus groups), quantitative (e.g. discrete choice experiments, best-worst scaling), or mixed methods
- EU regulators see value in robust PPS data to enhance understanding of patient perspectives, especially on preference-sensitive questions, e.g. balancing a clear benefit with severe or frequent side effects
- Important to continue developing foundational standards for PPS planning and conduct and guidance for integrating PPS results in decision-making
- The IMI PREFER framework: positive qualification opinion by CHMP as comprehensive reference for planning and conducting PPS
- Development of tools to assess and increase transferability of PPS results is encouraged

Methods to collect PED: patient engagement

- Patient engagement (PE) refers to all interactions with patients to gather their experience on their condition and/or priorities as to treatments and outcomes
- PE comprises a range of methods from involvement of individual experts and written consultations to surveys, focus groups, public hearings…
- EMA has an established framework for engaging with patients in regulatory activities
- Use of several complementary methods can enrich knowledge – but a single method (e.g. individual patients bringing their personal experience) should not be discounted
- Early engagement in medicines R&D can help e.g. to identify most appropriate methods (PROs, PPS…) to collect PED
- The 2022 CIOMS report on patient involvement in the development, regulation, and safe use of medicines is a high-level guide offering principles of good practice, case examples and recommendations for integrating PE in the medicine lifecycle

Transparency on how PED are assessed by regulators

- An updated template of the CHMP assessment report has been endorsed that better reflects different types of PED in a more structured way and explain how they have been used in the evaluation

2. Admin/regulatory information

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 Clinical efficacy

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>

6.3.2.1.3.4. <Secondary> objective<s>

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

Only include any additional objectives/goals that are directly relevant to the discussion and contribute to gaining further insights into the drug's performance.

Please describe also any objectives that include patient experience data (PED) that may be relevant.

<Text>

6.3.2.1.3.7. <Tertiary> objective<s>

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

Only include any additional objectives/goals that are directly relevant to the discussion and contribute to gaining further insights into the drug's performance.

Please describe also any objectives that include patient experience data (PED) that may be relevant.

<Text>

6.3.7. <Observational data><Data from registries>

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

If any Real-World Data (RWD), such as data from registries or observational data has been utilized by the applicant to support their claims, describe the specific data sources used and their intended purpose.

If RWD was employed to establish an external control arm this should be integrated into the corresponding pivotal study and detailed in the methods section of the assessment rather than in this section.

If patient experience data were collected as part of RWD, please describe it in this section.

<Text>

7. Risk Management Plan

7.4.2.3. <Patients engagement on the risk minimisation activities>

If patient experience data on risk minimisation preferences were submitted through third parties' consultation, include them here and explain if they were considered, and if not, why not.

10. Benefit-risk assessment

10.1.1. Disease or condition, <proposed> therapeutic indication

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

Provide a concise description of disease, disease epidemiology and available treatments essential for the BR evaluation. It should also cover any uncertainties and limitations in the current understanding of the condition.

- *If patient experience data has been submitted that relates to the disease or condition and its impact on daily life, functioning, etc. and is considered relevant, it should be mentioned (briefly!) in this section.*
- *Section 3.1 should not be repeated here (include a reference to section 3.1).*
- *At D80, D120, etc, present the proposed indication. By the end of the procedure, briefly summarise the agreed indication (or the CHMP's position on it, if an agreement cannot be reached – e.g. negative opinion). Please do not provide a comprehensive explanation of how the final indication was derived in this section but will be discussed in the balance section. This section is meant to be brief and simply an introduction to the BR.*

eCTD 2.5.6.1, 2.5.6.1.1, 2.5.6.1.2.

New template planned to be implemented in Q1 2025

Conclusions

- **EU regulators welcome PED** as important contribution to the totality of evidence and are working collaboratively to enable its broader use in regulatory decision-making
- **PED must be of high quality** to meet regulatory requirements
  - Scientific advice + qualification of novel methodologies
  - Contributing to methodological work and guidance/harmonisation via ICH
- **Increased transparency** on PED in the CHMP Assessment Report
- **Collaboration** is a key enabler:
  - Patient voice is critical throughout the lifecycle of medicines
  - Collaboration with other stakeholders – HTA, payers, healthcare providers is key
- **EMA reflection paper to be published for consultation in early 2025**

Thank you for your attention

Further information

Email: rosa.gonzalez-quevedo@ema.europa.eu

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

Follow us on **@EMA_News**