EUROPEAN MEDICINES AGENCY

Update on Patient Experience Data

Reflection paper

27 February 2024, Joint PCWP/ HCPWP

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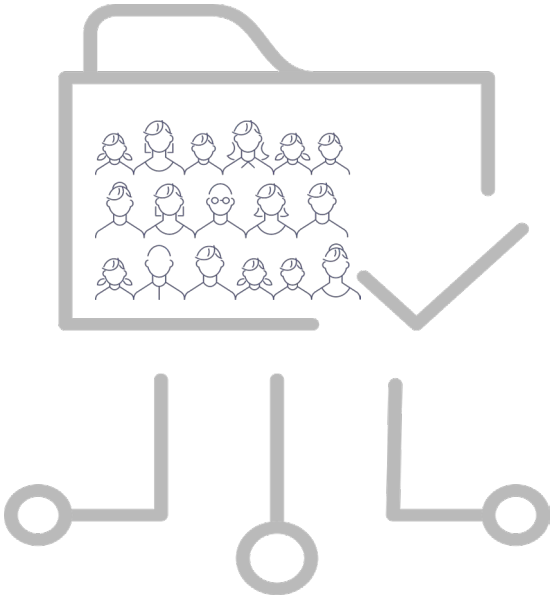
An agency of the European Union

Outline

- Patient Experience Data in the EU
- Update on progress
- PED Action plan
  - Reflection paper on EU approach to PED
- Conclusions

Patient Experience Data in the EU



- **Data reported directly by patients or their carers**, without interpretation by clinician
- **Reflects patient experience and preferences** of medicines and their views on their conditions
- **Proposal for an EU definition** as part of the EMA 2022 workshop
  - Definition **to be agreed with stakeholders**
  - **Types of patient experience data** in the EU:

Patient Reported Outcomes (PROs), Patient preference studies (PPS), Patient Engagement

Update on progress

√ 2022

EMA workshop on PED

√ 2023

PED cross-Agency group & Action Plan

√ 2023

PED Expert group

2024

Reflection Paper & Public Consultation

Update of Assessment Report

PED Action Plan

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 regulatory use cases	• Involvement of Patients in Big Data	• Collaborating experts
• List of priorities	• PCOs/HCPs - populating EMA data catalogues	• Mapping EU and international initiatives	• Update of Assessment Report	• PED research program including some RWD proof of concept studies	• EU Network training centre
• Monitor implementation	• Therapeutic area priorities	• Support HTA/payer contribution to reflection paper	• Exploring update of medicine overview and orphan medicines OMAR template	• Literature review of use of PED in non-interventional studies	• Coordinate stakeholder requests
• Network expert group		• Workshops on qualification, registries		• BDSG actions	• Overview of projects on PED with EMA involvement

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**
- **General EU framework or principles** – not a methodological guidance
- **Key action** derived from the 2022 PED workshop - requested by stakeholders
- Reflection paper is in the **Work Programmes of both CHMP and PRAC**

EU Network expert drafting group for PED reflection paper

- **Drafting group** set up with experts from the **EU Network**
- **Review by stakeholders and public consultation**
- **Timelines – 1st draft for public consultation expected in Q2 2024**

Membership	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: Oncology, Methodology, Scientific Advice, Big Data Steering Group, **PCWP, HCPWP**	Specific aspects to each working party (e.g. quality of life vs hard endpoints, methodology, qualification and SA, patient generated digital-data, stakeholders needs and perspectives)

Proposal for sections in the reflection paper (1)

Based on questions and requests from stakeholders

1. **Problem Statement / Scope**

2. **Definitions**

   PED, Patient Reported Outcomes, Patient Preference Studies, Patient Engagement, Patient Experience Evidence

3. **Use and value of PED along medicines' lifecycle and healthcare continuum**:
   - Medicines research and development
   - Initial benefit-risk decision-making
   - Orphan medicines designation
   - Post-marketing, ADR reporting and risk minimisation
   - HTA evaluations, reimbursement decisions and clinical care

Proposal for sections in the reflection paper (2)

4. PED Methodologies- PROs and other measures of clinical outcome

5. PED Methodologies - Patient preference studies

6. PED Methodologies - Patient Engagement

7. PED Sources:

- Digital PED
- Patient registries
- RWD and electronic health records
- European Health Data Space
- Safety monitoring databases

Proposal for sections in the reflection paper (3)

8. Challenges implementing PED

- Data quality, integrity and representativeness
- Data access (governance, privacy and security considerations)
- Other methodological challenges
- Guidance, legislative framework

9. EU approach to PED

- EMA Scientific advice and qualification of novel methodologies
- Global cooperation and ICH guidance
- Transparency on the use of PED in regulatory assessment

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)

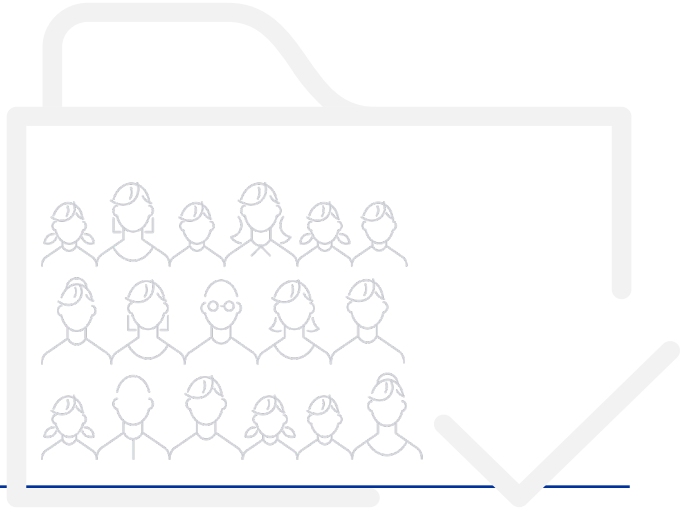
Qualification of novel methodologies

- **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

Next steps

- **Nomination of section leads and contributors** √
- **Drafting of the different sections** by leads and contributors - **ongoing** (end March 2024)
- **Section consolidation by EMA**
- **Drafting group written consultation**
- **Drafting group meeting**
- **Committees/Working party consultation & adoption**
- **Public consultation**

Thank you for your attention



Further information

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