EUROPEAN MEDICINES AGENCY

State of play and ongoing regulatory initiatives

Big Data Stakeholder Forum, Session 3: Patient Experience Data (PED)- realising the potential of PED in EU medicine regulation

4 December 2023

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An agency of the European Union

Outline

- Definition of Patient Experience Data (PED) in the EU
- PED as part of Big Data
- Status of PED in the EU
- Update on progress
  - PED Action plan
  - Upcoming reflection paper on EU approach to PED
  - ICH guidance
  - Transparency on how PED is assessed
  - Scientific advice & qualification of novel methodologies
- Conclusions

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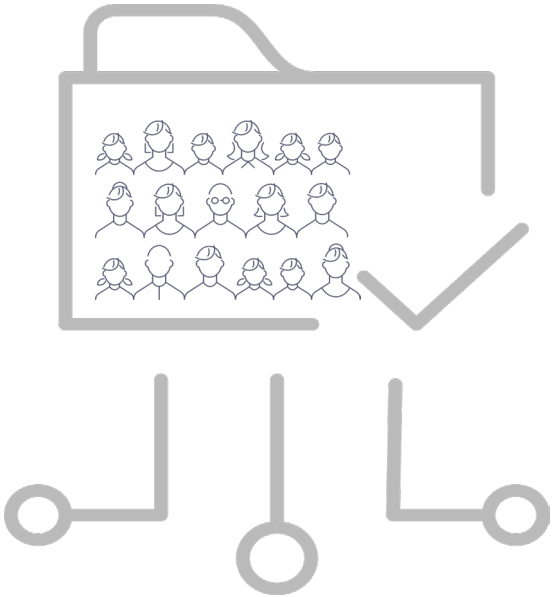
Definition of Patient Experience Data in the EU



- **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

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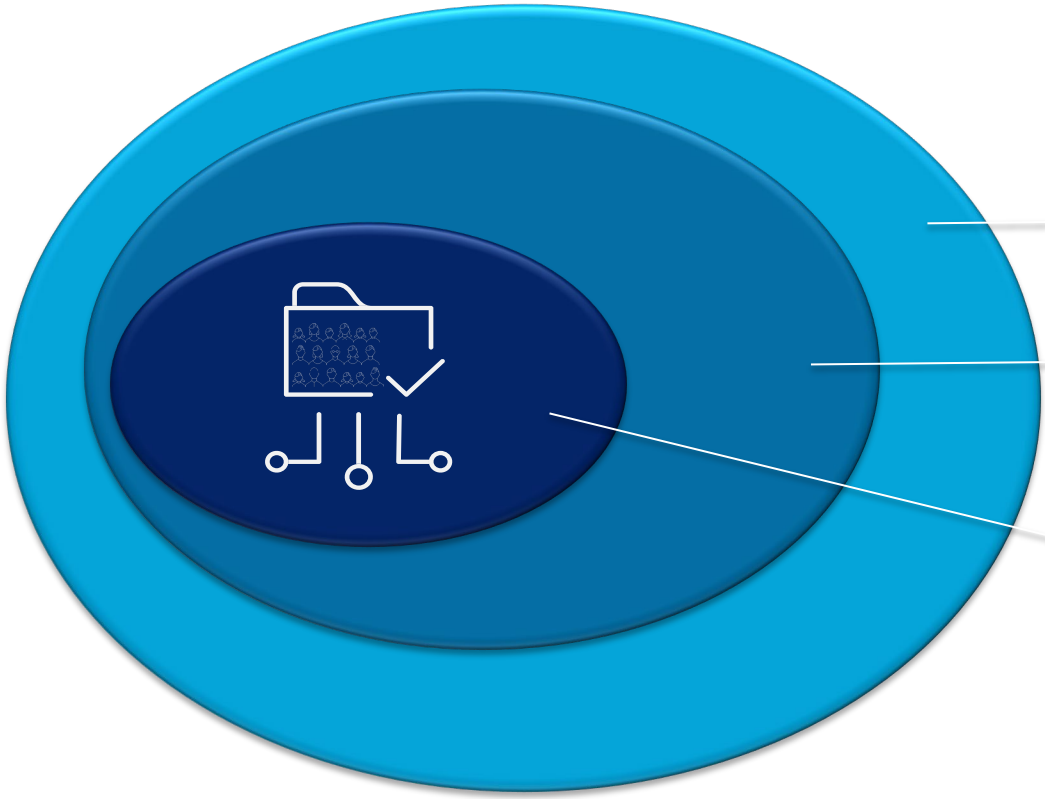
Patient Experience Data as part of Big Data



Big Data

Real World Data, Observational data (Patient Data)

Patient Experience Data (PROs, PPS, Patient engagement)

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Status of PED in the EU

- Reinforcing patient relevance in evidence generation is **key priority for the EU Network Strategy** and in EMA's **Regulatory Science Strategy**
- **Need for systematic inclusion of PED** in medicines development and regulation
- **PED is a new scientific discipline** – balance difficult methodological discussions with stakeholder engagement
  - Collaboration of multi-disciplinary experts cross-Agency and within EU Network
- **Opportunities for patient-generated digital data** thanks to novel technologies
- The **EU Network Strategy's delivery plan** and **CHMP's 2023 workplan** incorporate two key deliverables:
  - **Reflection paper** on the best EU approach to generate, collect and analyse PED
  - Explore how to **improve transparency in the Assessment Report**

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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

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

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Upcoming 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**

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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	Specific aspects to each working party (e.g. quality of life vs hard endpoints, methodology, qualification and SA, patient generated digital-data, etc)

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Proposal for elements to be included in reflection paper

Based on questions and requests from stakeholders

- Problem Statement
- Scope
- Definitions:
  PED, Patient Reported Outcomes, Patient Preference Studies, Patient Engagement, Patient Experience Evidence
- Use and value of PED along medicines' lifecycle and healthcare continuum
- Generation and collection of PED
- PED analysis
- Challenges/limitations
- **Scientific Advice & Qualification Novel Methodologies**
- Conclusions

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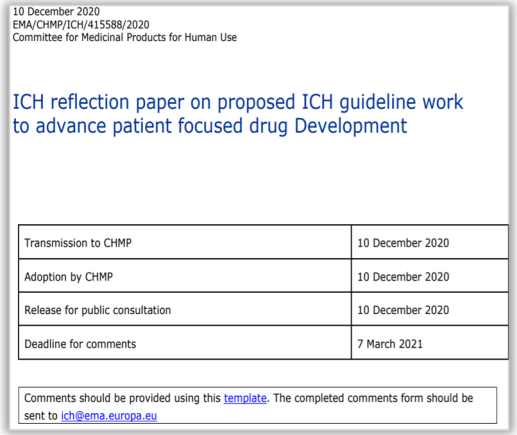
EU reflection paper to complement ICH Guidelines on PED

- Proposal for **new ICH guidelines** will provide **globally harmonized approach** to inclusion of **patient's perspective in a methodologically sound** way, to improve quality, relevance, safety and efficiency of drug development and to inform regulatory decision making.

  1) Focus on informing the drug development process, patient-reported outcomes

  2) Focus on patient preferences regarding benefits and risks

- Scope of Reflection Paper will differ from that of ICH guidance
  - Reflection paper will not cover specific methodological guidance



10 December 2020
EMA/CHMP/ICH/415588/2020
Committee for Medicinal Products for Human Use

ICH reflection paper on proposed ICH guideline work to advance patient focused drug Development

Transmission to CHMP	10 December 2020
Adoption by CHMP	10 December 2020
Release for public consultation	10 December 2020
Deadline for comments	7 March 2021

Comments should be provided using this template. The completed comments form should be sent to ich@ema.europa.eu

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Transparency on how PED are assessed

- **Stakeholders have called for more transparency** on how PED are used by regulators during the assessment of medicines.
- **Current Assessment Report** includes sections to reflect PED
- **EMA will review how to improve the Assessment Report** to ensure it covers all types of PED submitted by developers (e.g., PROs, PPS, Patient Engagement) and how they have been assessed and used in the evaluation (i.e. rationale for acceptance/exclusion in Benefit/Risk decision making).
- **How PED is reflected in the EU prescribing information** will be reviewed in the future.
- For orphan medicines, PED is also important for evaluating if a new medicine offers **significant benefit over existing treatments for patients suffering from rare diseases.** EMA will explore how to best reflect PED in the **Orphan Maintenance Assessment Report.**

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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

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Conclusions

- **PED is a new scientific discipline**
  - **Collaboration of multi-disciplinary experts** and stakeholders is needed
- **PED is an integral part of the Big Data work**
  - Leverage the **opportunities offered by digitalisation**
  - **Patients as integral part** of the Big Data Steering Group

- **EMA is working to progress on key PED deliverables**:
  - **Reflection paper** on the best EU approach to generate, collect and analyse PED
  - **Explore how to improve transparency** in the Assessment Report

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Thank you for your attention

Further information

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