Update on EMA progress on Patient Experience Data

Early Engagement to enhance the incorporation of Patient Experience Data in drug development programs and regulatory decision-making

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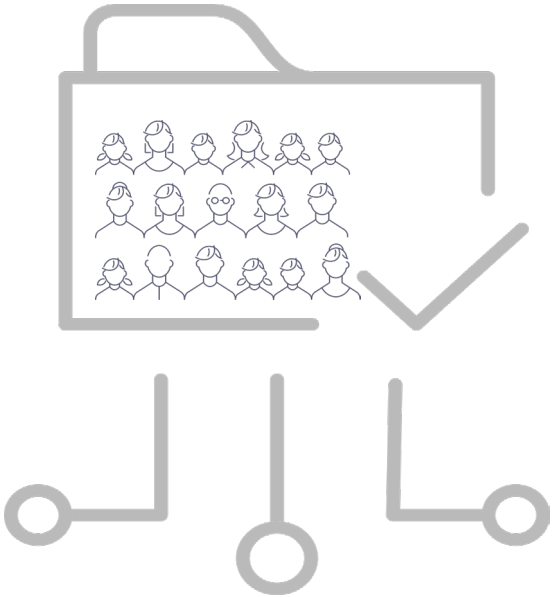
Public and Stakeholders Engagement Department

Disclaimer

The presenter does not have any conflict of interests.

Patient Experience Data in the EU



- **Data reported directly by patients or their carers**, without interpretation by clinician or third parties
- **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), data from Patient Engagement

(quantitative + qualitative data)

Status of PED in the EU

- **Reinforcing patient relevance in evidence generation** is a key priority in **EMANs** and the **Regulatory Science Strategy**
- Although there has been much progress in the EU in recent years, **PED are still not systematically included** in all aspects of medicines development and regulation
- **Stakeholder agreement on the relevance of PED** for medicines development and benefit-risk evaluation
- PED also relevant in the context of **implementation of the new EU Health Technology Assessment (HTA) regulation**, thus in **value assessments** by HTA and payers
- **Guidance work ongoing at ICH for global harmonisation** on PED
- **Stakeholder calls for progress and guidance** from EMA

Opportunities to further improve

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

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**
- **General EU framework or principles** – not a methodological guidance
- **Key action** derived from the 2022 PED workshop - requested by stakeholders

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 Q3 2024**

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: Oncology, Methodology, Scientific Advice, Big Data Steering Group, **Patients, Consumers and healthcare professionals' representatives**	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)

Elements to be covered in the reflection paper

1. **Problem Statement / Scope**
2. **Definition of PED**
3. **Use and value of PED along medicines' lifecycle and healthcare**
4. **PED Methodologies- Patient Reported Outcomes and other measures of clinical outcome, Patient Preference Studies, Patient Engagement**
5. **PED Sources**
6. **Challenges implementing PED**
7. **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

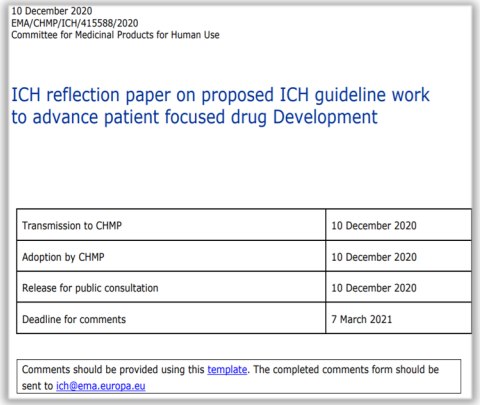
ICH Guidelines on PED

- 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
  - EU reflection paper to complement ICH 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

Transparency on how PED are assessed

- **Stakeholders have called for more transparency** on how PED are used by regulators
- **Current summary of evaluation (Assessment Report)** includes sections to reflect PED
- **EMA is reviewing how to improve the Assessment Report** to ensure it covers all types of PED and how they have been used in the evaluation
- **How PED is reflected in the EU prescribing information** will be reviewed in the future
- EMA will explore how to best reflect PED for orphan medicines, in the **Orphan Maintenance Assessment Report**

Conclusions

- EU regulators welcome patient experience data (PED) as an important contribution to the totality of evidence. We are working collaboratively to enable its broader use in regulatory decision- making. In this context, PED must be of high quality to meet regulatory requirements.

  → EMA working on an **EU Reflection paper**

  → **Scientific advice** + **qualification of novel methodologies**

  → More methodological work and **guidance and harmonisation (ICH)**

- **Transparency** to be improved:

  → Where PED is needed, **how it is collected and analysed**, and **how it is used**

- **Collaboration** is a key enabler:

  → **Patient's voice is critical** throughout the whole lifecycle of medicines

  → Collaboration with other stakeholders - **HTAs, payers, healthcare providers** - facilitate faster PED integration

Thank you for your attention

Further information

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