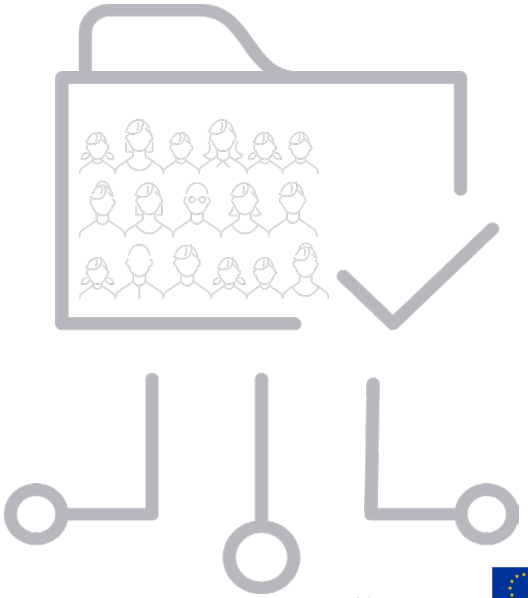
EUROPEAN MEDICINES AGENCY

Update on Patient Experience Data (PED)

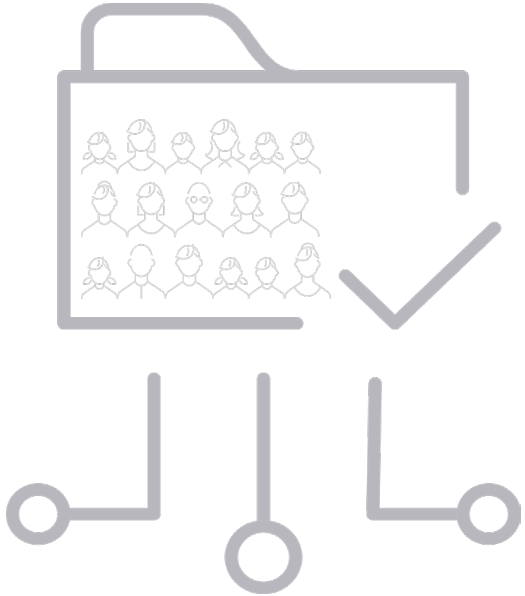
PCWP/HCPWP and all eligible organisations, 15 November 2023



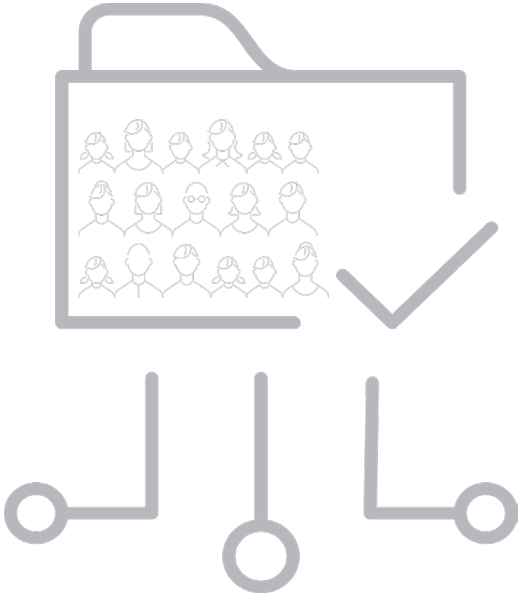
Presented by Rosa Gonzalez-Quevedo
Public and Stakeholders Engagement Department, EMA

Outline

- Patient Experience Data in the EU
- Highlights from June 2023 discussion
- Update on progress on PED in the EU
- Action Plan – List of priorities
- Reflection paper as key deliverable:
  - Expert drafting group for reflection paper
  - Multi-disciplinary expertise
  - Proposal for elements to be included in reflection paper
  - Drafting of reflection paper– next steps



Patient Experience Data in the EU



- Data reported directly by patients
- Reflects patient experience and preferences of medicines and their views on their conditions
- Proposal for an EU definition as part of the EMA workshop in 2022
  - Definition to be agreed with stakeholders

Highlights from June 2023 discussion

- **Need for systematic inclusion of PED** in medicines development and regulation
- PED is **key priority** for the EU Network and in EMA's **Regulatory Science Strategy**
- **PED is a new scientific discipline** – balance difficult methodological discussions with stakeholder engagement
  - **Collaboration of multi-disciplinary experts cross-Agency and EU Network is needed** to coordinate activities and provide content input into the deliverables
- **Opportunities for patient-generated digital data** thanks to novel technologies
- The **EMANS' 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 AR**
- **EMA has started work on the deliverables** by pooling cross-Agency expertise on PED

Update on progress on PED in the EU

√ 2022

EMA workshop on PED

√ 2023

PED cross-Agency group & Action Plan

√ 2023

PED Expert group

2024

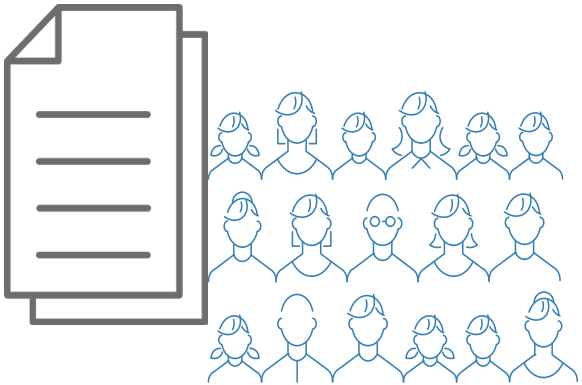
Reflection Paper & public consultation

Update of CHMP Assessment Report

Action Plan – List of priorities

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 use cases – scientific publications	• Involvement of PCOs in Big Data	• Collaborating experts
• List of priorities	• PCOs/HCPs - populating EMA data catalogues	• Mapping EU and international initiatives	• Update of AR template	• Proof of concept studies	• EU Network training centre
• Monitor implementation	• PCOs/HCPs -Data Quality Framework	• Support HTA/payer contribution to reflection paper	• Exploring update of medicine overview	• Literature review of use of PED in non-interventional studies	• Coordinate stakeholder requests
• Network expert group	• Therapeutic area priorities	• Workshops on qualification, registries	• Exploring update of OMAR template	• PED data sources in data catalogue	• Overview of projects on PED with EMA involvement
		• Ongoing projects	• Link to AI groups	• Learnings from ongoing SMA study	

Reflection paper as key deliverable of EU PED strategy



- **Key action** derived from the 2022 PED workshop - requested by stakeholders
- Reflection paper is in the **Work Programmes of both CHMP and PRAC**
- Sponsors: **Bruno Sepodes (CHMP) & Sabine Straus (PRAC)**

EU Network expert drafting group for PED reflection paper

- **Drafting group** being set up with experts from the **EU Network**
- **Experts' onboarding ongoing:**

  CHMP, PRAC, CMDh, PDCO, CAT, COMP, Methodology WP, Oncology WP, Scientific Advice WP, Big Data Steering Group
- Expert group may be involved in other deliverables such as **contribution to ICH guidelines**
- **Timelines –** 1st draft for public consultation expected in 2Q 2024

EU Network drafting group -multi-disciplinary expertise

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

Proposal for elements to be included in reflection paper

Based on questions and requests from stakeholders

- Problem Statement
- Scope
- Definitions: PED, PROs, PPSs, 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

EU reflection paper to complement and contribute to ICH Guidelines on PED

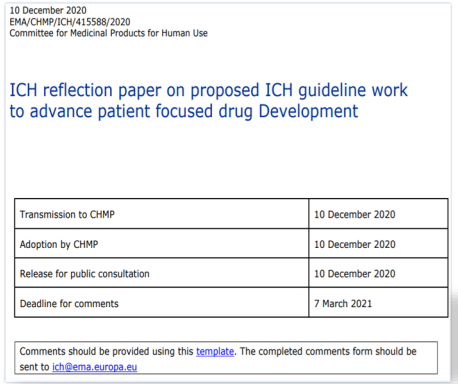
- Proposed **new ICH guidelines** will provide globally harmonized approach to inclusion of **patient's perspective in a methodologically sound** and sustainable way, with the aim to improve quality, relevance, safety and efficiency of drug development and to inform regulatory decision making.

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

  2) **Focus on the trade-offs between benefits and harms**

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

(avoid duplication)



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

Drafting of reflection paper– next steps

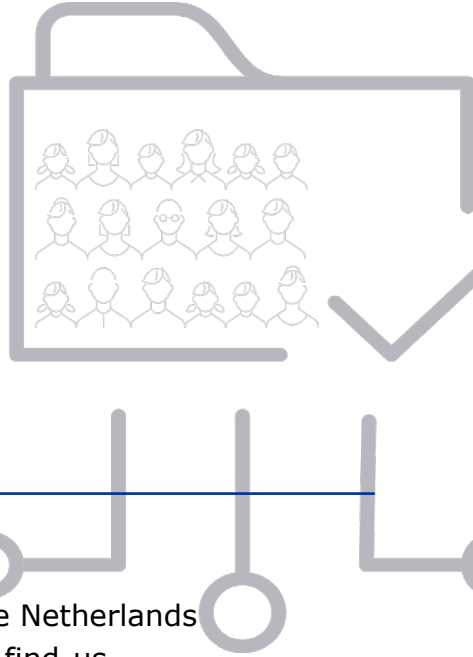
Establishment of EU Network drafting group

Drafting group kick-off meeting

Drafting process

Review by stakeholders and public consultation

Thank you for your attention



Further information

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