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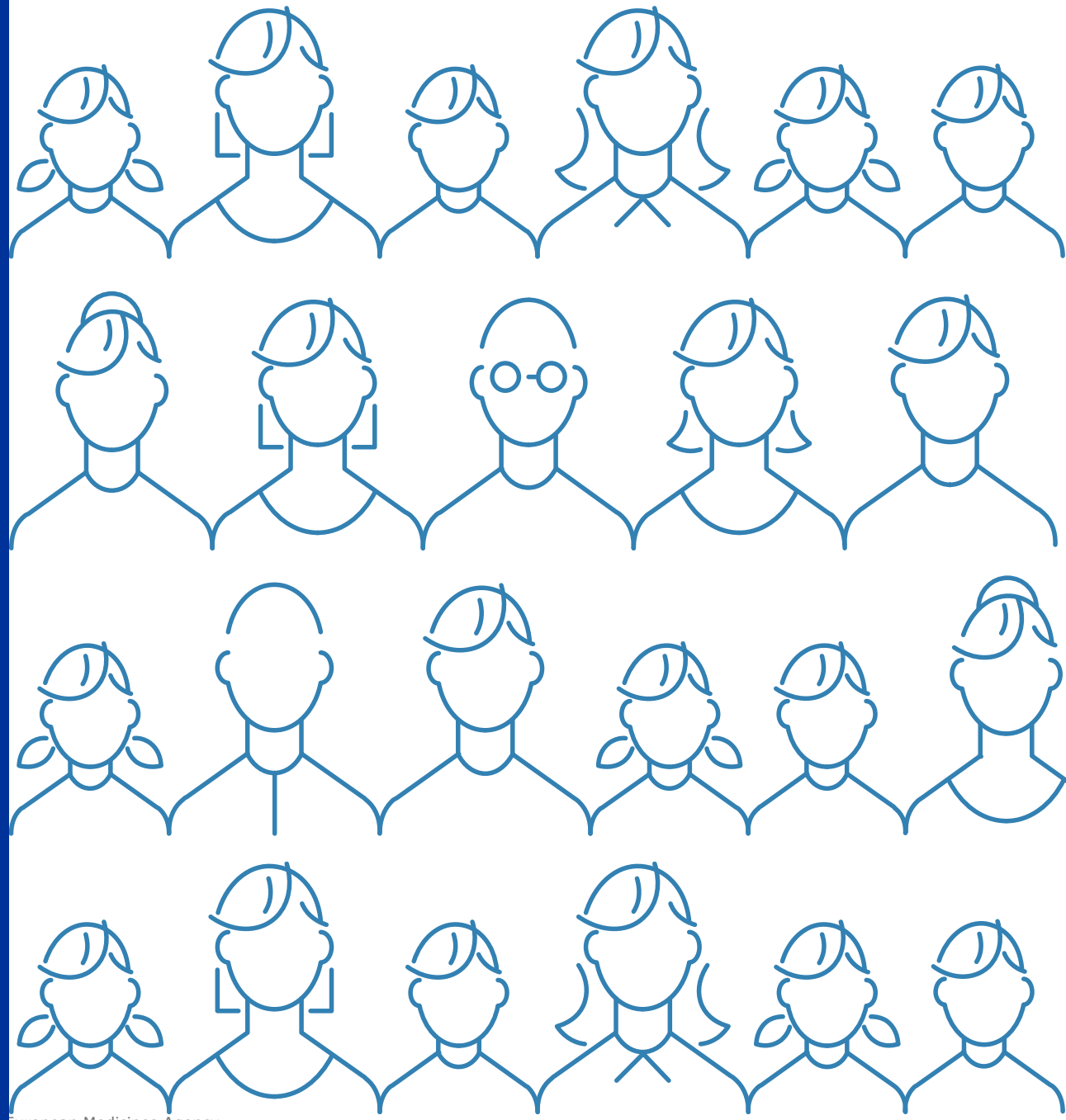
9.2. Update on Patient Experience Data – Reflection Paper

Presented by Kaisa Immonen

PCWP/HCPWP joint meeting, 1-2 April 2025



What is patient experience data (PED)

- **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 real world data (RWD) contexts

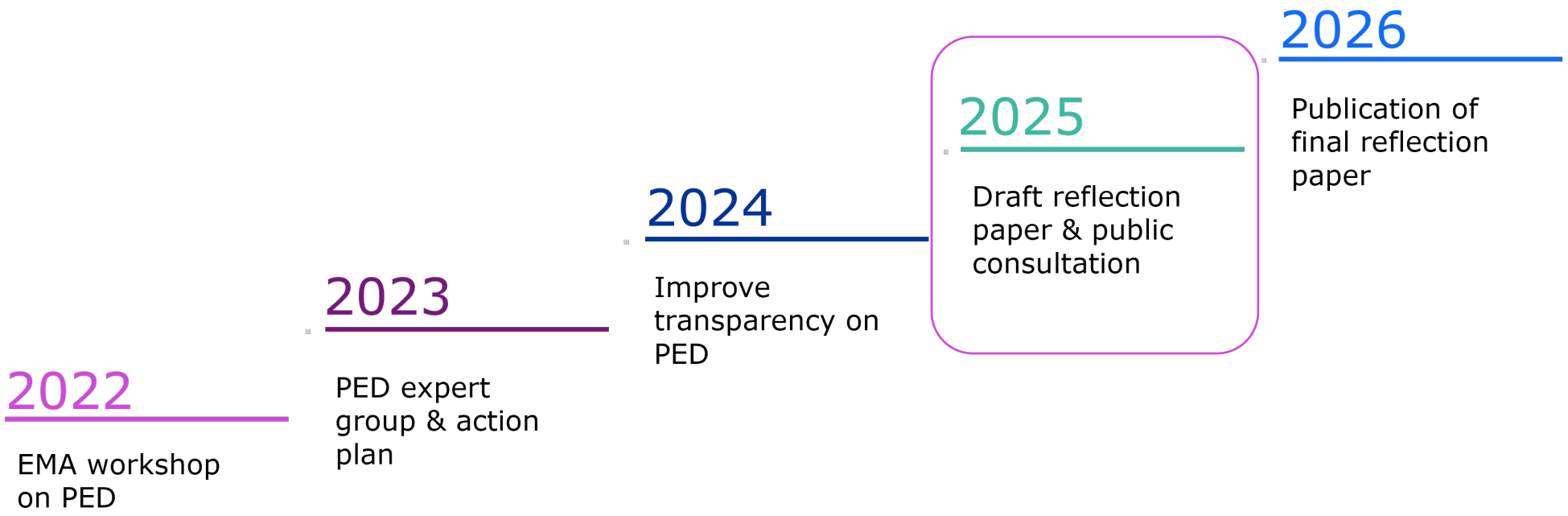
Patient experience data in the EU

- Reinforcing patient relevance in evidence generation is a key priority in the Regulatory Science Strategy and the European medicines agencies network strategy (See EMANS to 2025 and EMANS to 2028)
- 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 PED can be collected as part of RWD (e.g. registries, patient reports) to generate evidence

Opportunities to improve

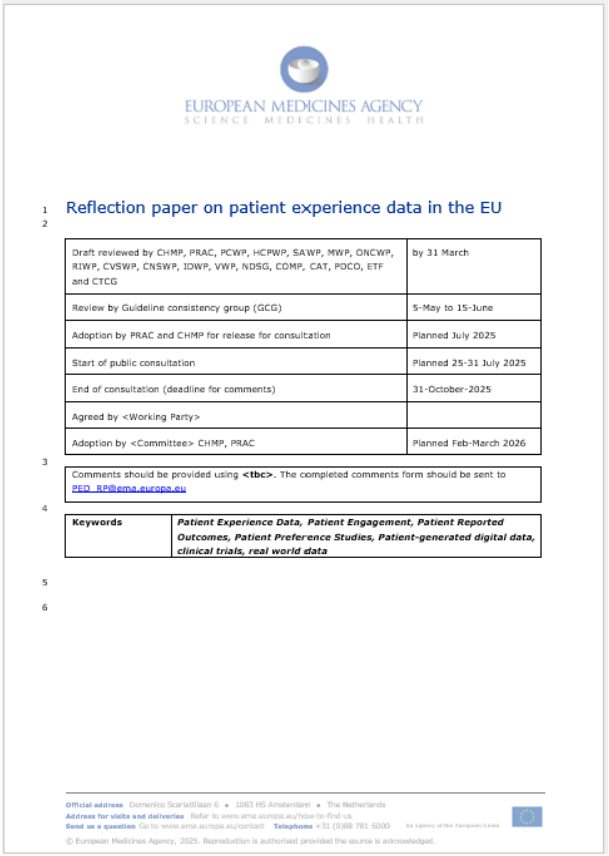
- Despite progress in recent years, PED are still not systematically included in all aspects of medicines development and regulation
- Stakeholder calls for progress and guidance from EMA to ensure the EU does not stay behind
- To optimise the use of PED, more work needed especially on:
  - Data collection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

Progress in PED



Reflection paper on EU approach to PED

- **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
- **Internal consultation** (incl. circulation to **PCWP/HCPWP**) in **March 2025**
- **Public consultation foreseen** in Q3 2025



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Reflection paper on patient experience data in the EU

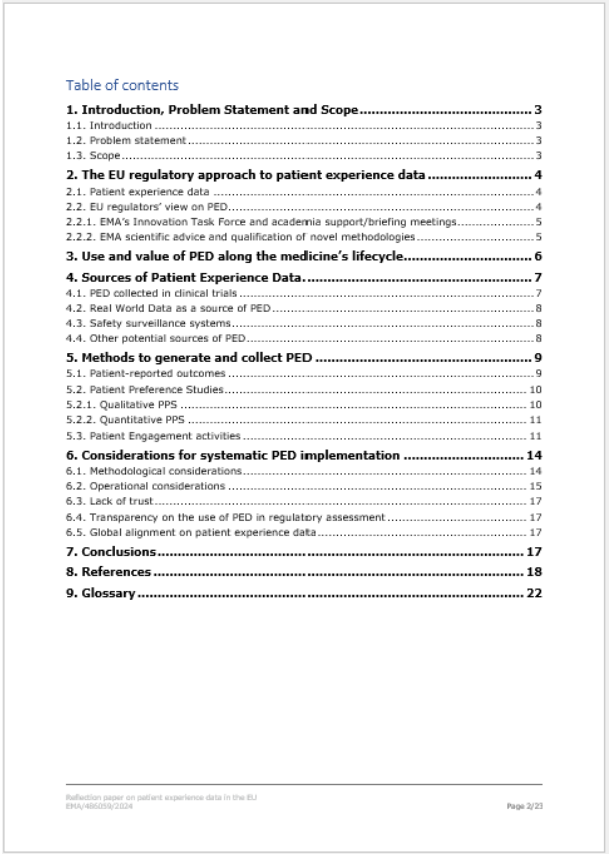
Draft reviewed by CHMP, PRAC, PCWP, HCPWP, SAWP, MWP, ONCWP, RIWP, CVSWP, CNSWP, IDWP, VWP, NDSG, COMP, CAT, PDCO, ETF and CTCG	by 31 March
Review by Guideline consistency group (GCG)	5-May to 15-June
Adoption by PRAC and CHMP for release for consultation	Planned July 2025
Start of public consultation	Planned 25-31 July 2025
End of consultation (deadline for comments)	31-October-2025
Agreed by <Working Party>	
Adoption by <Committee> CHMP, PRAC	Planned Feb-March 2026

Comments should be provided using **<tbc>**. The completed comments form should be sent to PED_RP@ema.europa.eu

Keywords	***Patient Experience Data, Patient Engagement, Patient Reported Outcomes, Patient Preference Studies, Patient-generated digital data, clinical trials, real world data***

Structure of the reflection paper

1. Introduction, Problem Statement and Scope
2. The EU regulatory approach to PED
   - Definition of PED
   - Scientific advice and qualification, Innovation Task Force, academia support
3. Use and value of PED along the medicine's lifecycle
4. Sources of PED (clinical trials, real-world settings)
5. Methods to generate and collect PED (PROs, PPS, patient engagement)
6. Considerations for systematic implementation (e.g. methodological, operational, acceptance/trust, global development)



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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
- **EMA reflection paper** to be published in **Q3 2025** for 3-month consultation
    - Comments already received from some PCWP/HCPWP members
    - Further input will be welcomed

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Thank you

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