Update on patient experience data

Cardiovascular Round Table, 20 February 2025

"Unmet Needs in Cardiovascular Diseases focusing on patient-benefit risk and patient-reported outcomes"

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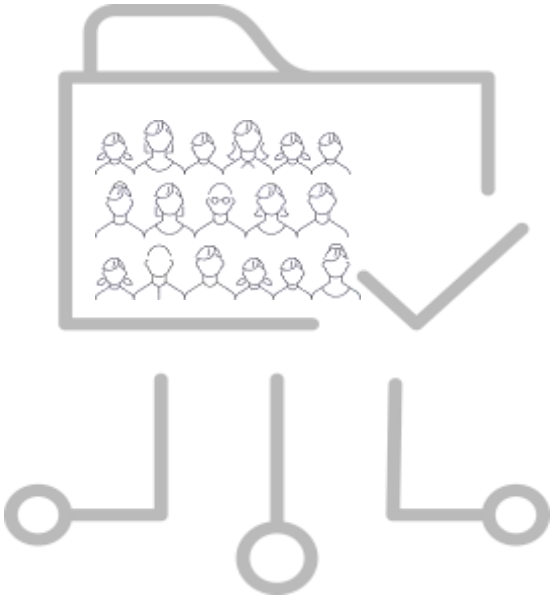
Disclaimer

The presenter does not have any conflict of interests.

Outline

- Patient experience data (PED) in the EU
- Relevance of PED for cardiovascular medicines
- Update on progress on PED
- EMA reflection paper on PED
- Conclusions

Patient experience data (PED) in the EU



- **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 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 draft EMANS to 2028)
- Patients' views on medicines or their condition are particularly important when quality of life can matter as much or more to patients than established endpoints (e.g. overall survival)
- 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 phase PED can be collected as part of RWD (e.g. registries, patient reports) to generate evidence

Opportunities to improve

- Although there has been progress in the EU 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 is needed especially on:
    - Data collection methods
    - Data quality and completeness
    - Methodologies applied to PED

Relevance of PED for cardiovascular medicines

- **Cardiovascular/metabolic** covers a broad area of diseases including CV Diseases, Diabetes and Obesity fields.
- **Hard endpoints required in these fields as main endpoints as per current regulatory guidelines given large populations to be treated and often life-long treatments however patients' views or preferences** on medicines or living with a condition falling within cardiovascular/metabolic diseases is gaining importance.
- Examples of PROs in recent developments (Camzyos)
- During the evaluation phase, collection of PED data by **reliable and validated** methodologies can contribute to the knowledge as **supportive data to primary or secondary endpoints.**
- In the post authorisation phase, PED can be collected **as part of RWD (e.g. in registries) to generate supportive evidence.**

Update on progress in PED

√ 2022

EMA workshop on PED

√ 2023

PED expert group & Action Plan

√ 2024

Improve transparency on PED

2025-26

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
- **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
- **Public consultation** foreseen in Q3 2025

Reflection paper on PED – elements to be covered

1. Problem statement and scope
2. EU approach to PED
    - Definition of PED
    - Scientific advice and qualification of novel methodologies, Innovation Task Force, Academia
3. Use and value of PED along the medicine lifecycle
4. Sources of PED (clinical trial and real-world settings)
5. Methods to collect PED (PROs, PPS, patient engagement)
6. Considerations for systematic PED implementation (e.g. methodological, data quality and access, acceptance/trust, global development)
7. Conclusion

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

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
- **Collaboration** is a key enabler:
  - Patient voice is critical throughout the lifecycle of medicines
  - Collaboration with other stakeholders – HTA, payers, healthcare providers is key
- **EMA reflection paper to be published in Q3 2025 for 3-month consultation**

Thank you for your attention

Further information

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Sources of PED

PED can be collected from a variety of sources:

- **Clinical trial settings**, e.g. PROs, patient preference data
  - Use of tailored, pre-planned research protocols for primary collection of PED in specific context of given trial is encouraged
- **Real-world settings**
  - Primary data collection – non-interventional studies – can support planned clinical trials e.g. by informing selection of patient-relevant endpoints
  - Secondary use of data – e.g. registries, EHRs, administrative databases – can together with other health data enhance information for individual patient management, research etc. – increasing efforts to integrate PED within secondary data sources
  - Patient reports of ADRs – pharmacovigilance
  - Not yet fully established: mHealth / wearables, digital devices, social networks

Methods to collect PED: patient-reported outcomes

- PROs are **health outcomes reported directly by the patient** about their health status without amendment or interpretation by a clinician or anyone else
- PROs capture **patient-relevant** disease- or treatment outcomes, e.g. symptoms, functioning, or general multidimensional concepts such as health-related quality of life (HRQoL) – captured through generic or specific measures
- PROs can **complement clinical outcome measures** – enhancing regulators' understanding of patients' experience, e.g. regarding symptoms, adverse effects → more accurate evaluation of benefits and risks
- **Challenges** include selection and validation of instruments, missing data, potential bias or uncertain clinical relevance
- Stakeholders developing PROs for use in regulatory decision-making can ask for **CHMP qualification opinion** or **parallel scientific advice** (HTA/EMA) or support from EMA's **Innovation Task Force**

Methods to collect PED: patient preference studies

- PPS not yet extensively used in regulatory decision-making – complex, time and resource intensive with few standardised methods – but **increasing interest** in their use in medicines development
  - ➢ Qualitative (e.g. interviews, focus groups), quantitative (e.g. discrete choice experiments, best-worst scaling), or mixed methods
- EU regulators see value in robust PPS data to **enhance understanding of patient perspectives**, especially on preference-sensitive questions, e.g. balancing a clear benefit with severe or frequent side effects
- Important to continue **developing foundational standards** for PPS planning and conduct and guidance for integrating PPS results in decision-making
- The IMI PREFER framework: *generally* positive qualification opinion by CHMP as comprehensive reference for planning and conducting PPS, *some limitations, case-by-case*
- Development of tools to assess and increase transferability of PPS results is encouraged

Methods to collect PED: patient engagement

- Patient engagement (PE) refers to all interactions with patients to gather their experience on their condition and/or priorities as to treatments and outcomes
- PE comprises a range of methods from involvement of individual experts and written consultations to surveys, focus groups, public hearings…
- EMA has an established framework for engaging with patients in regulatory activities
- Use of several complementary methods can enrich knowledge – but a single method (e.g. individual patients bringing their personal experience) should not be discounted
- Early engagement in medicines R&D can help e.g. to identify most appropriate methods (PROs, PPS…) to collect PED
- The 2022 CIOMS report on patient involvement in the development, regulation, and safe use of medicines is a high-level guide offering principles of good practice, case examples and recommendations for integrating PE in the medicine lifecycle