



EUROPEAN MEDICINES AGENCY
SCIENCE MEDICINES HEALTH

Multi-stakeholder workshop: **Patient experience data in medicines development and regulatory decision-making**





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21 September 2022, 10:00 – 16:30 (CEST), EMA, Amsterdam

Background and objectives

Patients have valuable insights and perspectives from living with a condition and its treatment. This includes symptoms, natural history, quality of life, unmet needs, which outcomes are important and preferences for future treatments. Input from patients, as users of medicines, can inform medicine development, enhance regulatory decision making and result in more patient-relevant outcomes.

EMA's Regulatory Science Strategy to 2025 recognises the need to identify optimal approaches for engaging patients in medicines development and benefit-risk assessments, including the development of standards for designing, conducting, analysing and reporting relevant studies incorporating patient experience data for regulatory submission, and to elucidate how such data can best inform regulatory decisions.

This multistakeholder workshop will bring together patients, healthcare professionals, academia, regulators, and industry to discuss ways to improve the collection and use of patient experience data to achieve patient-centred medicine development and regulation.

Workshop aims:

- Achieve a common understanding on what constitutes 'patient experience data', including patient engagement, patient preferences and patient reported outcomes.
- Reflect on current methods for collecting and incorporating patient data into medicines development and regulatory assessments
- Consider how direct patient data collection from real-world healthcare can be leveraged and used
- Agree on priorities to enhance the collection and use of patient experience data



Session 1: Patient Engagement

Chair: Harald Enzmann (CHMP)

- EMA framework for engagement (EMA)
- How patient engagement can contribute to the development and approval of medicines (patient)
- Survey results, proposed themes (industry)

Panel and audience discussion on values and limitations



Session 2: Patient Preference Elicitation

Chair: Bruno Sepodes (CHMP)

- How patient preferences can contribute to development and regulation of medicines (academic)
- Considerations and learnings from use-cases (industry)
- Patient Preference research (patient)

Panel and audience discussion on values and limitations



Session 3: Patient Reported Outcomes

Chair: Peter Mol (UMCG)

- PRO contribution to the development and approval of medicines (academic)
- PRO data generation in practice (CHMP)
- PRO tools (industry)

Panel and audience discussion on values and limitations



Session 4: Digitalisation for patient-generated health data

Chair: Peter Arlett (EMA)

- European Health Data Space (EC)
- Tools to collect patient generated data (academic)
- Data platforms (patient)

Panel and audience discussion on values and limitations



Session 5: Guidance on collection and use of patient data

Chair: Spiros Vamvakas (EMA)

- Qualification of novel methodologies (EMA)
- Patient experience data in decision making and future guidance (industry)
- ICH Patient-focused drug development (PFDD) initiative (EMA)

Panel and audience discussion on values and limitations



Session 6: Summary and next steps

Chair: Juan Garcia-Burgos (EMA)

- Summary and recommendations from each session
- Panel and audience discussion