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

21 September 2022, 09:45 – 16:45 (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.

The aims of the workshop are to:

- 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

Multi-stakeholder workshop on patient experience data in medicines development and regulatory decision-making

Chaired by Juan Garcia-Burgos (EMA)

09:30 Joining and technical checks

09:45 Welcome and opening speech

Welcome and workshop objectives **5'**
Emer Cooke (EMA)

Opening speech:

What is 'patient experience data' and why it is valuable **10'**
Steffen Thirstrup (EMA)

10:00 Session 1: Patient Engagement

Chair: Harald Enzmann (BfArM/CHMP)

EMA framework for engagement **5'**
Maria Mavris (EMA)

How patient engagement can contribute to the development and approval of medicines **10'**
Yann Le Cam (EURORDIS)

How patients contribute to the safety monitoring of medicines **10'**
Sabine Straus (MEB/PRAC)

Survey results, proposed themes **10'**
Lucia D'Apote (EUCOPE)

Panel and audience discussion on values and limitations **25'**
Additional panellists:
Kaisa Immonen (EPF/PCWP)
Ulrich Jager (EHA/HCPWP)

11:00 Session 2: Patient Preference Elicitation

Chair: Bruno Sepodes (UL/INFARMED/CHMP)

How patient preferences can contribute to development and regulation of medicines **10'**
Jorien Veldwijk (ESHPM)

Considerations and learnings from use-cases **10'**
Mireille Muller (EFPIA)

Patient Preference research **10'**
Kate Morgan (MPE)

Panel and audience discussion on values and limitations **30'**
Additional panellists:
Francesco Pignatti (EMA)
Maggie Galbraith (HAS)

12:00 *Coffee break*

12:10 Session 3: Patient Reported Outcomes

Chair: Peter Mol (UMCG/MEB)

PRO contribution to the development and approval of medicines **10'**
André Elferink (MEB/SAWP)

PRO data generation in practice **10'**
Carla Torre (UL)

Clinical Outcome Assessment (COA) Implementation and Utility **10'**
Ebony Dashiell-Aje (EuropaBio)

Panel and audience discussion on values and limitations **30'**
Additional panellists:
Martin Huber (BfArM/PRAC)
Beate Wieseler (IQWiG)
Joke Jaarsma (EFNA)
Milton Bonelli (EMA)

13:10 *Lunch*

13:50 Session 4: Digitalisation for patient-generated health data

Chair: Jesper Kjaer (DKMA/BDSG)

European Health Data Space **10'**
Jerome De Barros (EC)

Tools to collect patient generated data **10'**
Tanja Stamm (MUV)

Data platforms **10'**
Elizabeth Vroom (WDO)

Panel and audience discussion on values and limitations **30'**
Additional panellists:
Carla Torre (UL)
Marilena Vrana (EHN)
Thorsten Vetter (EMA)
Koenraad Batselier (COCIR)

14:50 Session 5: Guidance on collection and use of patient data

Chair: Spiros Vamvakas (EMA)

Qualification of novel methodologies **10'**
Thorsten Vetter (EMA)

Patient experience data in decision making and future guidance **10'**
Susan Bhatti (EFPIA)

ICH Patient-focused drug development (PFDD) initiative **10'**
Milton Bonelli (EMA)

Q&A **15'**

15:35 *Coffee break*

15:45 Session 6: Summary and next steps

Chair: Juan Garcia-Burgos (EMA)

Summary and recommendations from each session **25'**
Session chairs

Panel and audience discussion on recommendations and priorities **30'**
Panellists:
Harald Enzmann (BfArM/CHMP)
Sabine Straus (MEB/PRAC)
Bruno Sepodes (UL/INFARMED/CHMP)
Peter Mol (UMCG/MEB)
Jesper Kjaer (DKMA/BDSG)
Spiros Vamvakas (EMA)

16:40 Closing remarks

Wrap up **5'**
Juan Garcia-Burgos (EMA)

16:45 End of meeting

List of chairs, speakers and panellists

Koenraad Batselier	Representative of the European Coordination Committee of the Radiological, Electromedical and Healthcare IT Industry (COCIR)
Susan Bhatti	Representative of the European Federation of Pharmaceutical Industries and Associations (EFPIA)
Milton Bonelli	Scientific specialist, Scientific Advice Office, European Medicines Agency (EMA)
Yann Le Cam	CEO, Rare Diseases Europe (EURORDIS)
Emer Cooke	Executive Director, European Medicines Agency (EMA)
Lucia D'Apote	Representative of the European Confederation of Pharmaceutical Entrepreneurs (EUCOPE)
Ebony Dashiell-Aje	Representative of the European Association for Bioindustries (EuropaBio)
Jerome De Barros	Policy Officer, Digital Health, European Reference Networks, European Commission (DG SANTE)
André Elferink	Dutch Medicines Evaluation Board (MEB), Member of EMA's Scientific Advice Working Party (SAWP) and Vice chair of Central Nervous System Working Party (CNSWP)
Harald Enzmann	Head of licensing division in the Federal Institute for Drugs and Medical Devices, Germany (BfArM), Chair of Committee for Medicinal Products for Human Use (CHMP)
Maggie Galbraith	Project manager, HTA division, French National Authority for Health (HAS)
Juan Garcia-Burgos	Head of Public and Stakeholders Engagement Department, European Medicines Agency (EMA)
Martin Huber	Head of unit PRAC, Legal Status, Adverse Reaction Reports, Medication Errors Federal Institute for Drugs and Medical Devices (BfArM), Vice-chair of the Pharmacovigilance Risk Assessment Committee (PRAC)
Kaisa Immonen	Director of Policy at the European Patients' Forum (EPF), former co-chair of Patients' and Consumers' Working Party (PCWP)
Joke Jaarsma	President of European Federation of Neurological Associations (EFNA) and Vice chair of Restless Legs Foundation, Netherlands
Ulrich Jager	European Hematology Association (EHA), former co-chair of Healthcare Professionals Working Party (HCPWP), Medical University of Vienna

Jesper Kjaer	Director of Data Analytics Centre, Danish Medicines Agency, Co-chair HMA / EMA Big Data Steering Group (BDSG)
Maria Mavris	Liaison specialist, Public and Stakeholder Engagement department, European Medicines Agency (EMA)
Peter Mol	Professor of Drug Regulatory Science University Medical Center Groningen (UMCG), Dutch Medicines Evaluation Board (MEB)
Kate Morgan	Head of Policy and Access, Myeloma Patients Europe (MPE)
Mireille Muller	Representative of the European Federation of Pharmaceutical Industries and Associations (EFPIA)
Francesco Pignatti	Head of Oncology and haematology, European Medicines Agency (EMA)
Bruno Sepodes	University of Lisbon (Portugal), Portuguese National Authority for Medicines and Health Products (INFARMED) and Vice chair of EMA's Committee for Medicinal Products for Human Use (CHMP)
Tanja Stamm	Head of Section for Outcomes Research, Center for Medical Statistics, Informatics and Intelligent Systems, Medical University of Vienna
Sabine Straus	Chair of Pharmacovigilance Risk Assessment Committee (PRAC), Medicines Evaluation Board (MEB)
Steffen Thirstrup	Chief Medical Officer, European Medicines Agency (EMA)
Carla Torre	Co-opted member of EMA's Committee for Medicinal Products for Human Use (CHMP), University of Lisbon (Portugal)
Spiros Vamvakas	Scientific Adviser on Human Medicines, European Medicines Agency (EMA)
Jorien Veldwijk	Assistant Professor, Erasmus School of Health Policy and Management (ESHMP)
Thorsten Vetter	Scientific specialist, Scientific Advice Office, European Medicines Agency (EMA)
Marilena Vrana	Manager of Patients and Research at the European Heart Network (EHN), member of EMA's Patients and Consumers Working Party and HMA/EMA Big Data Steering Group
Elizabeth Vroom	Chair of World Duchenne Organization (WDO), Director of Duchenne Parent Project, Netherlands, member of EMA's Patients and Consumers Working Party (PCWP) and DARWIN advisory board
Beate Wieseler	Head of Department Drug Assessment at the Institute for Quality and Efficiency in Health Care (IQWiG)