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
  Emer Cooke (EMA) 5’

- Opening speech:
  What is ‘patient experience data’ and why it is valuable
  Steffen Thirstrup (EMA) 10’

10:00  Session 1: Patient Engagement

Chair: Harald Enzmann (BfArM/CHMP)

- EMA framework for engagement
  Maria Mavris (EMA) 5’

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

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

- Survey results, proposed themes
  Lucia D’Apote (EUCOPE) 10’

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

11:00  Session 2: Patient Preference Elicitation

Chair: Bruno Sepodes (UL/INFARMED/CHMP)

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

<table>
<thead>
<tr>
<th>Name</th>
<th>Organization and Role</th>
</tr>
</thead>
<tbody>
<tr>
<td>Koenraad Batselier</td>
<td>Representative of the European Coordination Committee of the Radiological, Electromedical and Healthcare IT Industry (COCIR)</td>
</tr>
<tr>
<td>Susan Bhatti</td>
<td>Representative of the European Federation of Pharmaceutical Industries and Associations (EFPIA)</td>
</tr>
<tr>
<td>Milton Bonelli</td>
<td>Scientific specialist, Scientific Advice Office, European Medicines Agency (EMA)</td>
</tr>
<tr>
<td>Yann Le Cam</td>
<td>CEO, Rare Diseases Europe (EURORDIS)</td>
</tr>
<tr>
<td>Emer Cooke</td>
<td>Executive Director, European Medicines Agency (EMA)</td>
</tr>
<tr>
<td>Lucia D’Apote</td>
<td>Representative of the European Confederation of Pharmaceutical Entrepreneurs (EUCOPE)</td>
</tr>
<tr>
<td>Ebony Dashiell-Aje</td>
<td>Representative of the European Association for Bioindustries (EuropaBio)</td>
</tr>
<tr>
<td>Jerome De Barros</td>
<td>Policy Officer, Digital Health, European Reference Networks, European Commission (DG SANTE)</td>
</tr>
<tr>
<td>André Elferink</td>
<td>Dutch Medicines Evaluation Board (MEB), Member of EMA’s Scientific Advice Working Party (SAWP) and Vice chair of Central Nervous System Working Party (CNSWP)</td>
</tr>
<tr>
<td>Harald Enzmann</td>
<td>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)</td>
</tr>
<tr>
<td>Maggie Galbraith</td>
<td>Project manager, HTA division, French National Authority for Health (HAS)</td>
</tr>
<tr>
<td>Juan Garcia-Burgos</td>
<td>Head of Public and Stakeholders Engagement Department, European Medicines Agency (EMA)</td>
</tr>
<tr>
<td>Martin Huber</td>
<td>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)</td>
</tr>
<tr>
<td>Kaisa Immonen</td>
<td>Director of Policy at the European Patients’ Forum (EPF), former co-chair of Patients' and Consumers' Working Party (PCWP)</td>
</tr>
<tr>
<td>Joke Jaarsma</td>
<td>President of European Federation of Neurological Associations (EFNA) and Vice chair of Restless Legs Foundation, Netherlands</td>
</tr>
<tr>
<td>Ulrich Jager</td>
<td>European Hematology Association (EHA), former co-chair of Healthcare Professionals Working Party (HCPWP), Medical University of Vienna</td>
</tr>
</tbody>
</table>
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)