Joint HMA/EMA multi-stakeholder workshop on Patient Registries

12-13 February 2024

In-person at the EMA building, Amsterdam + virtual enabled

This joint two-day hybrid HMA/EMA workshop on Patient Registries follows on the successful disease-specific workshops held between 2017 and 2019, as well as the EMA multi-stakeholder workshop on qualification of novel methodologies.

The event will bring together representatives of registry holders, regulatory agencies, pharmaceutical companies, patients, healthcare professionals, academia, and health technology assessment bodies to address the following objectives:

- Day 1 - 12 February afternoon: Discuss the EMA qualification procedure for patient registries with the aim to identify the benefits, current limitations, and propose measures to optimise the process;
- Day 2 - 13 February all day: Establish the value and enable the use of patient registries for regulatory decision-making by considering contexts of use for which registry data are 'fit for purpose'. We will also explore mechanisms to support data characterisation, discoverability, and assessment, including key topics for a 'feasibility assessment' template to evaluate the relevance of a registry in view of a specific study question and design.

Monday, 12 February – First day of Workshop

Chaired by Peter Arlett (EMA, Head of Data Analytics and Methods Task Force (TDA)) and Patricia McGettigan (Queen Mary University Of London, PRAC Independent Scientific Expert, BDSG member)

Objectives: Discuss the EMA qualification procedure for patient registries[1]

- Identify the benefits of registry qualification.
- Identify current limitations and gaps, as well as possible measures to optimise the procedure.
- Propose a check list to guide the structure and content of qualification applications.
- Explore opportunities for strengthened collaboration and effective mechanisms to ensure standards of qualified registries are sustained over time.

12:30 Joining and technical checks

13:00 Welcome and opening remarks

Welcome, objectives and structure of the day — 10'

Peter Arlett (EMA, Head of TDA) and Patricia McGettigan (PRAC, BDSG member)

13:10 Session 1: Scene setting on the EMA qualification procedure

Co-Chairs: Bruno Sepodes (INFARMED, CHMP Vice-Chair) and Alexis Nolte (EMA, Head of Human Division)

Introduction by the Chairs — 10'

Bruno Sepodes (INFARMED, CHMP Vice-Chair) and Alexis Nolte (EMA, Head of Human Division)

Main lessons learnt so far on the qualification of registries — 45'

Registries perspective: Lutz Nährlich (European Cystic Fibrosis) and Jan Hillert (Big Multiple Sclerosis Data Network)

Industry perspective: Gracy Crane (Roche)

Regulator perspective: Sabine Straus (MEB, PRAC Chair)

Q&A — 20'

Explanation on breakout sessions — 15'

Carla Jonker (EMA, TDA - Real World Evidence)

[1] In according with the EMA Guideline on registry-based studies, "Patient registry" (referred to as "registry" in the rest of the agenda) is defined as: Organised system that collects uniform data (clinical and other) to identify specified outcomes for a population defined by a particular disease, condition or exposure. The term 'patient' highlights the focus of the registry on health information. It is broadly defined and may include patients with a certain disease, pregnant or lactating women or individuals presenting with another condition such as a birth defect or a molecular or genomic feature.

14:40 Session 2: Breakout sessions (onsite participants only)

Breakout session A: Benefits of the qualification of patient registries, limitations, reaching common understanding on stakeholders' expectations, measures to optimise the process.

Breakout session B: Draft check list for the qualification application to guide the structure and content of requests, and other means to ease the procedure.

Breakout session C: Mechanisms to ensure standards of qualified registries are sustained over time (post-qualification lifecycle process).

16:35 Coffee break and move to plenary room

17:00 Session 3: Feedback from breakout sessions and recommendations on next steps

Co-Chairs: Paolo Foggi (AIFA, SAWP Chair) and Iordanis Gravanis (EMA, Head of Scientific Advice)

Panel discussion 50'

3 nominees to report on the breakout sessions and discussion with all participants.

17:50 Closing remarks

Wrap up 10'

Peter Arlett (EMA, Head of Data Analytics and Methods Task Force) and Patricia McGettigan (PRAC, BDSG member)

Tuesday, 13 February – Second day of Workshop

Chaired by Peter Arlett (EMA, Head of Data Analytics and Methods Task Force (TDA)) and Patricia McGettigan (Queen Mary University of London, PRAC Independent Scientific Expert, BDSG member)

Objectives: Establish the value and enable the use of patient registries for regulatory decision-making

- Examine contexts of use for which registry data are 'fit for purpose' and demonstrate the value of patient experience data across the spectrum of regulatory use cases.
- Share experience on addressing challenges to foster the use of registries for regulatory purposes, through enhanced description of data quality, increased data discoverability, and promotion of data interoperability.
- Consider the key elements of a 'feasibility assessment' to evaluate the relevance of a registry in view of a specific study question and design.
- Identify areas for collaboration between stakeholders to strengthen the use of registries for regulatory purposes and decision making.

08:30 Joining and technical checks

09:00 Welcome and opening speeches

Welcome to the workshop 5'
Peter Arlett (EMA, Head of Data Analytics and Methods Task Force)

Opening remarks from the EMA Executive Director 5'
Emer Cooke (EMA Executive Director)

Opening remarks from a Member State 5'
Peter Mol (MEB, CHMP)

Opening remarks from the European Commission (EC) 5'
Simona Martin (Directorate General Joint Research Centre) and Christina Kyriakopoulou (Directorate General Research and Innovation)

09:20 Session 1: Status quo on registries for regulatory purposes

Co-Chairs: Peter Mol (MEB, CHMP), Francesca Day (EMA, Head of Therapeutic Areas)

What are the regulatory needs in terms of registry data? 20'
Regulator: Bruno Sepodes (INFARMED, CHMP)

Use case highlighting opportunities and challenges of registries for regulatory decision-making 50'
Study on Spinal Muscular Atrophy disease (SMA)
Regulator: Kieran Breen (Parkinson's Europe, CAT member)
Aetion: Nicolas Deltour
TREAT-NMD: Seung Lee, Neil Bennett
Patient representative: Mencia de Lemus Belmonte (SMA Europe, CAT member)
Industry: Simon Bennett (Biogen)

Q&A 30'

11:00	Coffee break	
11:30	Session 2: Experience gained, and lessons learnt from initiatives aiming to leverage the use of registries	
	Co-Chairs: Sabine Straus (MEB, PRAC Chair), Patrice Verpillat (EMA, Head of TDA - Real World Evidence)	
	Fit for purpose and Data Quality Framework *Kit Roes (MEB, Radboud UMC Nijmegen, MWP Chair)*	20'
	Data discoverability: EMA catalogues on RWD sources and studies *Ana Cochino (EMA, TDA - Healthcare Data), Franz Schaefer (ERN ERKNet)*	20'
	Interoperability between registries and other data sources: HARMONY Big Data Platform *Jesús María Hernández Rivas (Institute of Biomedical Research of Salamanca, IBSAL)*	20'
	Q&A	20'
12:50	Session 3: "Fit for purpose" registries breakout sessions (onsite participants only)	
	Explanation on breakout sessions *Kelly Plueschke (EMA, TDA - Real World Evidence)*	15'
13:05	Lunch	
14:00	Session 3: "Fit for purpose" registries breakout sessions (onsite participants only)	

Breakout session A (2 parallel groups A1 and A2): Foster the use of patient registries for regulatory decision-making through enhanced description of data quality, increased data discoverability, and promotion of data interoperability (e.g. Data quality framework (DQF) for EU medicines regulation and its chapter on Real-World Data (RWD), EMA catalogues).

Breakout session B (2 parallel groups B1 and B2): Key elements of a 'feasibility assessment' to evaluate the relevance of a registry in view of a specific study question and design, areas for collaborations to strengthen the use of registries for regulatory purposes and decision-making.

16:00	Coffee break	

16:30 Session 4: Feedback from breakout sessions and recommendations on next steps

Chair: Patricia McGettigan (PRAC, BDSG member)

Panel discussion — 50'

4 nominees to report on the breakout sessions and discussion with all participants.

17:20 Closing remarks

Wrap up — 10'

Patrice Verpillat (EMA, Head of TDA - Real World Evidence)

Acronyms

AIFA	Agenzia Italiana del Farmaco, Italy
BDSG	Big Data Steering Group
CAT	Committee for Advanced Therapies
CHMP	Committee for Medicinal Products for Human Use
EC	European Commission
EMA	European Medicines Agency
ERK Net	European Rare Kidney Disease Reference Network
ERN	European Reference Network
HMA	Heads of Medicines Agency
INFARMED	Autoridade Nacional do Medicamento e Produtos de Saúde I.P., Portugal
MEB	Medicines Evaluation Board, The Netherlands
MWP	Methodology Working Party
PRAC	Pharmacovigilance Risk Assessment Committee
SAWP	Scientific Advice Working Party
SMA	Spinal Muscular Atrophy
TDA	Data Analytics and Methods Task Force