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 tools 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) and Patricia McGettigan (Queen Mary University Of London, PRAC Independent Scientific Expert, BDSG member)

Objectives: Discuss the EMA qualification procedure for patient registries

- 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 mechanism 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
Peter Arlett (EMA) and Patricia McGettigan (PRAC, BDSG member)

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

Co-Chairs: TBC (EMA Committee Chair) and Alexis Nolte (EMA, Head of Human Division)

Introduction by the Chairs

Main lessons learnt so far on the qualification of registries

Registries perspective: Combined presentation on behalf of the concerned registries (European Cystic Fibrosis, EBMT, Enroll-HD, TREAT-NMD, Big MS Data Network, International Niemann-Pick Disease Registry, World Federation of Haemophilia Gene Therapy Registry, HARMONY BD platform)

Industry perspective: Gracy Crane (Roche)

Regulators perspective: Sabine Straus (MEB, PRAC Chair)

Q&A

Overview of feedback received on preparatory work
Ana López de la Rica Manjavacas (AEMPS, BDSG)

Explanation on breakout sessions
Carla Jonker (EMA)

1 In accordance 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:** Identify the benefits of the qualification of patient registries, reach common understanding on stakeholders’ expectations.

**Breakout session B:** Discuss a draft check list for qualification application to guide the structure and content of requests, and other tools or processes to ease the procedure.

**Breakout session C:** Explore 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), Iordanis Gravanis (EMA, Head of Scientific Advice)*

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

17:50  **Closing remarks**

**Wrap up**  
*Peter Arlett (EMA) 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) 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)

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

Opening remarks from a Member State 5’
Member State representative (TBC)

Opening remarks from the European Commission 5’
European Commission representative (TBC)

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: (TBC)

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 (AETION)
TREAT-NMD: Seung Lee, Neil Bennet
Patient representative: Mencia de Lemus Belmonte (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-RWE)

**Fit for purpose and Data Quality Framework**  
Kit Roes (MEB, Radboud UMC Nijmegen, MWP Chair)  
20’

**Data discoverability: EMA catalogues or RWD sources and studies**  
Ana Cochino (EMA), 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  
15’

12:45  Session 3: “Fit for purpose” registries breakout sessions (onsite participants only)

**Overview of feedback received on preparatory work**  
Christine Dehn (Deutsche Herzstiftung e.V.)  
15’

**Explanation on breakout sessions**  
Kelly Plueschke (EMA)  
5’

13:05  Lunch

14:00  Session 3: “Fit for purpose” registries breakout sessions (onsite participants only)

**Breakout session A:** Tools to improve feasibility assessment and identify “fit for purpose” registries: Data quality framework (DQF) for EU medicines regulation and its chapter on Real-World Data (RWD), as well as EMA catalogues.

**Breakout session B:** 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
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