EUROPEAN MEDICINES AGENCY
SCIENCE MEDICINES HEALTH

3 September 2020
EMA/428155/2020

# Agenda - Workshop on the draft guideline on registry-based studies

**19 October 2020**

**12:30 – 17:00 (CET)**

**Virtual meeting**

**Official address** Domenico Scarlattilaan 6 ● 1083 HS Amsterdam ● The Netherlands
**Address for visits and deliveries** Refer to www.ema.europa.eu/how-to-find-us
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## Background

Many registry-based studies are performed to support regulatory decision-making in the pre- or post-authorisation setting. In order to address methodological aspects of studies conducted in registries to generate scientific evidence related to medicinal products, the EMA Cross-Committee Task Force on Registries has drafted a Guideline on registry-based studies.

The main objective of the draft Guideline is to provide recommendations on key methodological aspects of registry-based studies and the relevant legal basis and regulatory requirements for marketing authorisation applicants and holders. It is also relevant to patients and to those involved in the funding, creation and management of registries, those participating in the collection and analysis of registry data, and those planning to use the registry information and infrastructure to perform registry-based studies with a possible regulatory purpose.

The document defines a registry-based study as an investigation of a research question using the infrastructure of a new or existing registry for patient recruitment and data collection. A patient registry is defined as an organised system that collects data and information on a group of people defined by a particular disease, condition, exposure or health-related service, and that serves a pre-determined scientific, clinical and/or public health (policy) purpose.

An Annex of the draft Guideline reviews aspects of patient registries that regulators consider good practice for their use for regulatory purposes in general and more specifically registry-based studies.

The workshop will be broadcast live on EMA's website. You can follow the broadcast by clicking on the 'multimedia' tab on the event page on the day of the event. In addition to the online broadcast, there is limited capacity to participate in the virtual meeting room for people who wish to make verbal contributions to the meeting.

## Objectives

- Respond to key questions and requests for clarification;
- Present stakeholders' perspectives on the usefulness of the Guideline for registry-based studies;
- Present recent experience on methodological aspects of registry-based studies.

## Agenda

| Item | Topic | Speaker | Time |
|---|---|---|---|
| 1. | Connection to virtual room and technical checks | - | 12.30 |
| 2. | - Welcome and introduction<br>- Workshop objectives<br>- Opening remarks | - Peter Arlett<br>- Xavier Kurz<br>- Peter Mol | 12.45 |
| **SESSION 1:**<br>**Overview of core recommendations of the draft Guideline and comments received**<br>***Chair: Peter Mol*** | | | |
| 3. | Overview of the main recommendations of the draft Guideline on registry-based studies | Xavier Kurz | 13.00 |
| 4. | Summary of the comments received from stakeholders by October 9th, 2020 | Valerie Strassmann | 13.15 |
| 5. | Plenary discussion, Questions and Answers | All | 13.30 |
| **BREAK – 14.00** | | | |
| **SESSION 2:**<br>**Stakeholders' perspective on the use of the Guideline for registry-based studies**<br>***Chair: Sabine Straus*** | | | |
| 6. | - Regulator<br>- Regulator<br>- Registry holder<br>- Pharmaceutical industry<br>- Patients association | - Milena Stain<br>- Marion Haberkamp<br>- Eoin McGrath<br>- Chris Chinn<br>- Mariette Driessens | 14.15 |
| 7. | Plenary discussion and summary | All | 15.15 |
| **BREAK – 15.40** | | | |
| **SESSION 3:**<br>**Recent experience on methodological aspects of registry-based studies**<br>***Chair: TBD*** | | | |
| 8. | Experience of randomisation within a registry | Barbara Casadei | 15.50 |
| 9. | Data quality and data verification in registries: results of a stakeholders' survey | Carla Jonker | 16.05 |
| 10. | Collection of safety information linked to medicinal products through registries: results of a survey among registries listed in the ENCEPP Resources database | Kelly Plueschke | 16.20 |
| 11. | Plenary discussion | All | 16.35 |
| 12. | Summary of meeting and next steps | Peter Mol | 16.50 |
| **End of meeting – 17.00** | | | |

## Speakers

Carla Jonker – Medicines Evaluation Board (MEB - NL)

Chris Chinn – Co-Chair of the Integrated Evidence Generation and Use Working Group, EFPIA

Barbara Casadei - President of the European Society of Cardiology, EuroHeart Registry

Eoin McGrath – European Society for Blood and Marrow Transplantation (EBMT)

Kelly Plueschke – Data Analytics Workstream, EMA

Mariette Driessens – Patient Alliance for Rare and Genetic Diseases (VSOP); Patients Network for Medical Research and Health (EGAN)

Marion Haberkamp – Federal Institute for Drugs and Medical Devices (BfArM – DE); SAWP member

Milena Stein – EMA Committee for Medicinal products for Human use (CHMP) member; Bundesamt für Sicherheit im Gesundheitswesen (BASG - AT)

Peter Arlett – Head of Data Analytics and Methods task Force, EMA

Peter Mol – Chair of the EMA Cross-Committee Task Force on Registries; Medicines Evaluation Board (MEB – NL)

Sabine Straus – Chair of the EMA Pharmacovigilance and Risk Assessment Committee (PRAC); Medicines Evaluation Board (MEB - NL)

Valerie Strassmann – Data Analytics Workstream, EMA

Xavier Kurz – Head of Data Analytics Workstream, EMA