Joint HMA/EMA Big Data Steering Group Workshop on RWE methods: Harnessing Real-World Data for Regulatory Use

14 June 2024 : 09.00 AM-16.30 PM

Hybrid meeting / EMA, Amsterdam

The objectives of the workshop are:

- Hear the views of stakeholders and experts:
  - on the draft RWE reflection paper open for public consultation in May
  - on priorities for further regulatory guidance development and collaboration beyond the reflection paper.
- Engage with stakeholders on novel RWE methods in regulatory decision making.

Target Audience:

- Internal: EMA staff and European Medicines Regulatory Network (EMRN)
- External: Representatives from pharmaceutical industry, academia, regulatory bodies, health technology assessment (HTA), patients and healthcare professionals (HCP) by providing insights on use of RWE for regulatory decision making.

Format:

- One day hybrid workshop (online and in person by invitation)

Friday, 14 June 2024, 09:00 – 16:30 CEST

Chaired by Peter Arlett (EMA, Head of Data Analytics and Methods Task Force (TDA), BDSG Co-chair) and Jeppe Larsen (Danish Medicines Agency, BDSG Co-chair)

08:45 Joining and technical checks (for online)

09:00 Welcome

Opening remarks from EMA
Peter Arlett (EMA BDSG Co-chair) – 5 min

Opening remarks from HMA
Jeppe Larsen (HMA BDSG Co-chair) – 5 min

Scene-setting and goals of the workshop
Patrice Verpillat (EMA, Head of RWE) – 10 min

EU-funded initiatives in the use of new RWE methods
Tomasz Dyla (DG Research and Innovation) – 10 min

09:30-10:50 Session 1: Presentation and discussion of RWE Reflection paper

Chaired by Kit Roes (MWP Chair) and patient representative (TBD)

Presentation of the RWE Reflection paper - 40 min
Xavier Kurz (ESEC RWE)

Panel discussion with invited stakeholders from pharmaceutical industry, academia, regulatory bodies, HTA, patients and HCP *(Ioana Agache)* - 40 min
Moderator: Olaf Klungel

10:50-11:15 Coffee Break

11:15-12:45 Session 2a: RWE methods to support EU regulatory decision making

Chaired by Sabine Straus (PRAC) and TBD (invited Harald Enzmann)

Target Trial Emulation and Estimand frameworks for Non-interventional Studies with causal objectives

Introduction – 15 min
Xabier Garcia de Albeniz (RTI Barcelona)

Presentations of Target Trial Emulation in a regulatory context – 45 min
- *Juan Jose Abellan Andres (EMA)*
- *Daniel Prieto Alhambra (DARWIN CC)*
- *Industry use case (invite through EMA's Stakeholder Engagement Department)*

Panel discussion with panellists from pharmaceutical industry, academia, regulatory bodies, HTA – 30 min

12:45-13:30 Lunch

13:30-15:00 Session 2b: RWE methods to support EU regulatory decision making

Chaired by Carla Torre (CHMP) and Marcia Rueckbeil (EMA)

RWD-derived External Controls in Clinical Trials

Introduction – 15 min
Elina Asikanius (SAWP, MWP)

Presentations of RWD as external control in a regulatory context – 45 min

- *Industry use case (invite through EMA's Stakeholder Engagement Department)*
- *FDA use case - Donna Rivera (FDA)*
- *EMA use case (Abecma) – joint presentation by Andrea Buzzi (EMA) + TBD (Methodology ESEC)*

Panel discussion with panellists from pharmaceutical industry (*Mehmet Burcu (MSD)*)**, academia, regulatory bodies, HTA** – 30 min

15:00-15:20 Coffee Break

15:20-16:20 Session 3: The next three years: Roadmap for RWE guidance

Chaired by Jeppe Larsen (BDSG Co-chair) and TBD (MWP/EMA Chair)

Introduction to Methodology Working Party – 15 min
Kit Roes (MWP Chair)

MWP Roadmap for the development of RWE guidance – 15 min
Olaf Klungel (MWP Member)

Panel discussion with invited stakeholders from pharmaceutical industry, academia, regulatory bodies, HTA, patients and HCP *(Holger Schunemann)* – 30 min

16:20-16:30 Summary of the workshop and conclusion

Concluding remarks and Wrap up
Peter Arlett (EMA BDSG Co-chair)