EUROPEAN MEDICINES AGENCY
SCIENCE MEDICINES HEALTH

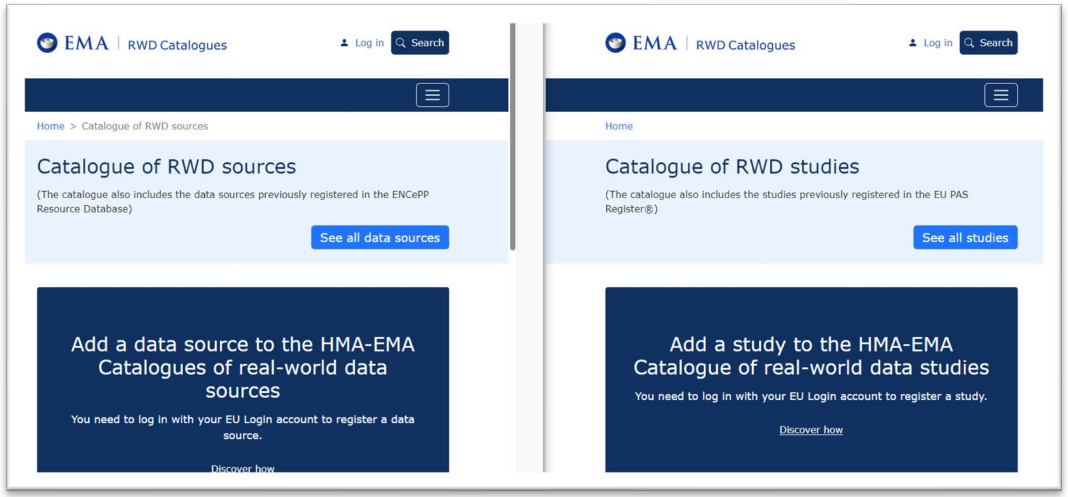
Focus group

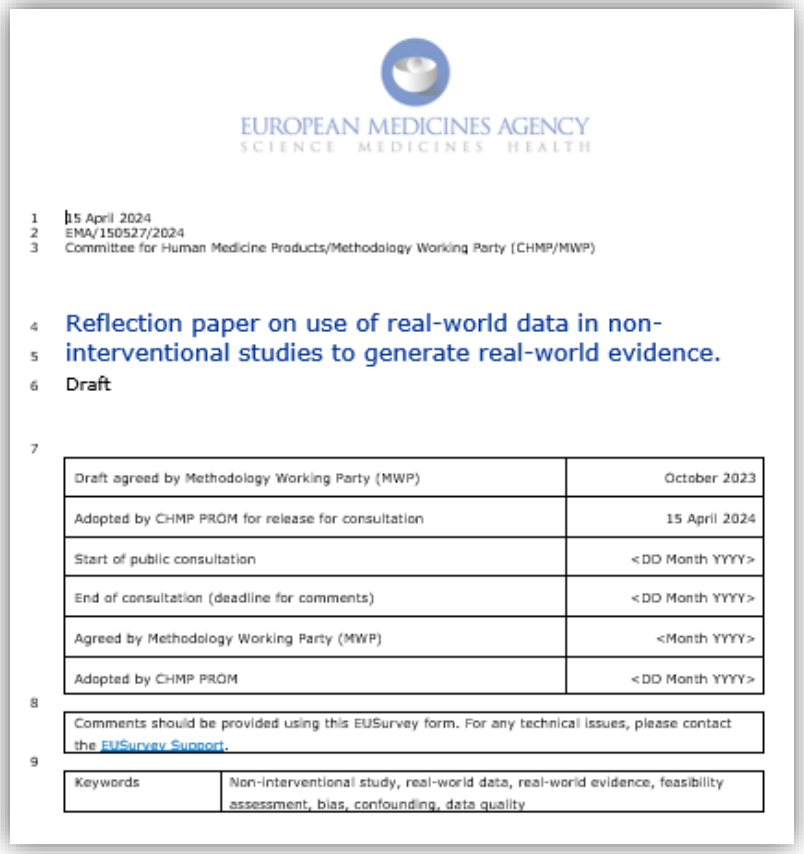
on use of RWD and generation of RWE

Background, Objectives & Working Methods

Patrice Verpillat

An agency of the European Union

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15 April 2024
EMA/150527/2024
Committee for Human Medicine Products/Methodology Working Party (CHMP/MWP)

Reflection paper on use of real-world data in non-interventional studies to generate real-world evidence.

Draft

Draft agreed by Methodology Working Party (MWP)	October 2023
Adopted by CHMP PROM for release for consultation	15 April 2024
Start of public consultation	<DD Month YYYY>
End of consultation (deadline for comments)	<DD Month YYYY>
Agreed by Methodology Working Party (MWP)	<Month YYYY>
Adopted by CHMP PROM	<DD Month YYYY>

Comments should be provided using this EUSurvey form. For any technical issues, please contact the EUSurvey Support.

Keywords	Non-interventional study, real-world data, real-world evidence, feasibility assessment, bias, confounding, data quality

Willingness to intensified dialogue and continued collaboration on RWD/RWE

Overall objective of the Focus Group

Share knowledge and experience with use of RWD and generation of RWE to advance integration of relevant and reliable RWE in regulatory decision making

Specific objectives

To **exchange views on RWD and RWE key concepts, discuss priorities and possible approaches, explore possible solutions** for enhancing the use of excellent evidence generated by the analysis of RWD into regulatory decision making

- The Focus Group should particularly discuss any relevant topics related to design of studies using RWD (e.g., data quality, fit-for-purpose data, feasibility assessment, place for innovative designs and/or methods…), interpretation and reporting of results (e.g., impact and interpretation of heterogeneity in multi-database studies…), and good science principles to be applied to any study using RWD (e.g., transparency, fit-for-purpose evidence…)
- The Focus Group could also identify RWD/RWE areas for multi-stakeholder approach needs, if relevant

Focus Group

For **EFPIA** (European Federation of Pharmaceutical Industries and Associations)

- **Álmath Spooner**, Head of Europe Regulatory Policy and Intelligence at AbbVie
- **Gracy Crane**, International Regulatory Science Policy Lead (RWD & Data Policy) & RWD Chapter Lead at Roche
- ***Sigrid Behr***, Group Head Quantitative Safety & Epidemiology at Novartis - *Alternate*

For **EUCOPE** (European Confederation of Pharmaceutical Entrepreneurs)

- **Andrew Cooper**, Vice President Global Head of Epidemiology & Real World Evidence at Shionogi
- **Milada Mahic**, Director of the RWE Global Centre of Excellence at Vertex Pharmaceuticals
- ***Marieke Schoonen***, Senior Director Observational Research at Amgen - *Alternate*

Focus Group

For **EuropaBio**

- **Mark Marsico**, MSD
- **Lisa Hampson**, Novartis

For **Vaccines Europe**

- **Carol Koro**, Vice President and Head of Vaccines Epidemiology, Global Regulatory, Safety & Quality at GSK
- **Sylvia Taylor**, Head of Medical Evidence for Vaccines and Immune Therapies Unit at AstraZeneca
- ***Mark Marsico***, Senior Director & Research Head, RWE Policy and Health Equity Research at MSD - *Alternate (note that Mark Marsico has also been proposed as a delegate by EuropaBio)*

For **AESGP** (European Self-Care Industry association)

- **Fernando Raúl Urrutia-González**, Schwabe
- ***Abir Tadmouri Sellier***, Pierre Fabre - *Alternate*

Kick-Off meeting on September 25

4 (most probably) **meetings per year** to allow time to work in between on some of the topics

- **Duration** to be adapted: 50 minutes may be too short, rather plan for 80 minutes (to be shortened pending agenda items)
- **Agenda** to cover a limited number of topics to have time to discuss and possibly come to a decision (anticipated to have 1 to 2 topics per meeting max). Some topics may need more than one call (meeting). Equally, preparation ahead will be critical (explaining the proposed way of working shared on the slides -> call for agenda items a month in advance or even decided at the end of the previous call, so that everyone during the following call can talk on behalf of their respective trade associations and not on behalf of individual companies. We will see all along if this needs to be adapted)

No publication expected from this group's work. Discussions and outputs will be reported twice per year during R&D platform meetings (-> support operational aspects linked to RWD/E with concrete deliverables)

Topics for the next meeting (Dec 03)

EFPIA & EUCOPE

Topic 1: Progress of the Q&A document

Topic 2: Processes around generation and use of RWE via DARWIN EU

- Better understand the triggers behind initiation of different studies in DARWIN EU - Who proposes different types of the studies and what is the purpose?
- Level of involvement of MAH in a study: would it be possible for EMA to keep MAH more involved or at least informed when the study is initiated (regardless of the type), protocol publicly available, and report finalized? MAH often has years of experience and knowledge regarding their products and could add valuable input on data sources, study design and interpretation of the results
- Lack of transparency about the use of the study report among health authorities, agency itself , HTA bodies…

Topics to be covered during the meeting early 2025

EFPIA

Topic 1: DARWIN-EU feasibility

Use DARWIN-EU studies to discuss principles for feasibility assessments (including the assessment of relevance of the data - as per EMA DQF). The DARWIN-EU website (Study Execution) shares only high-level information about feasibility assessments and it’s not clear if these assessments follow a framework or any other structure

- What are the core elements that should be addressed during an initial feasibility assessment? What is the role of the PICO framework to ensure a systematic approach for the feasibility assessment? How might the target trial framework be used in feasibility assessments? How could a milestone approach be applied to feasibility assessments, given that the assessment is likely to be iterative? Which principles should be applied for conducting feasibility assessments (e.g. Define clear decision criteria for what is considered "feasible" / "infeasible")? How does the feasibility assessment relate to the data quality dimension "relevance"?