

Insights into Stakeholders Needs - Workshop Protocol

The Real4Reg Consortium aims to ensure high visibility of the project and the promotion of active interaction with key stakeholders. As stated in the Dissemination, Exploitation and Communication Plan, Real4Reg “*will organise three workshops for RWD experts to address the needs from early career researchers to experienced professionals, and also the lay public, including patients. Registration will be free-of-charge to encourage the participation and training of young and junior team members in the partner organisations.*”

The second workshop took place in October 2024, the details are provided below.

Workshop Title: Real4Reg - Insights into Stakeholder Needs

Date: 14th October

Time: 09:30 - 11:00 CEST

Online Platform: WebEx

Agenda:

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|-------------|--|
| 09:30-09:35 | Introduction |
| 09:35-09:50 | Keynote Presentation: EMA insights on RWE and training needs - Luca Giraldi (European Medicines Agency) |
| 09:50-10:00 | Real4Reg survey results: Lucas Depner (DZNE) ¹ |
| 10:00-10:55 | Roundtable session – discussion of the Real4Reg survey results and how they can inform training programmes
Moderation: Vera Ehrenstein (Aarhus University)
Participants: <ul style="list-style-type: none">Luca Giraldi (European Medicines Agency)Niklas Hedberg (Heads of HTA Agencies Group);Gianmario Candore (Bayer)Carla Torre (Faculty of Pharmacy of the University of Lisbon)Paulo Gonçalves (RD-Portugal) |
| 10:55-11:00 | Closing |

¹ DZNE - German Center for Neurodegenerative Diseases

The workshop was publicised through email invitations sent to a list of stakeholders, as well as via publications on the Real4Reg website, social media, and promotion by Real4Reg partners on their websites and social media channels.

Participants:

The workshop had a total of 108 participants from 14 European countries. Participants came from different types of organisations: regulatory agencies/HTA, universities, industry companies, payers, healthcare institutions, patient associations, among others. Participants held a wide range of professional roles such as scientists/researchers, market access specialists, project managers, pharmacovigilance officers, among others.

The audience also included members of the Real4Reg consortium and advisory board, members of other sister projects of the MetReal Cluster, and related institutions.

Workshop recording and materials

The recording of the workshop is available on Real4Reg website, as well as the presentations of the speakers. (Click [here](#))

Summary of the activities:

Keynote Presentation: Real-world evidence & training needs: a regulatory perspective - Luca Giraldi (European Medicines Agency)

Outline:

- EMA vision and activities on RWE
- Main issues regulators face with RWE
- Big Data Training Curriculum for regulators

Presentation: Real4Reg survey results: Lucas Depner (DZNE)

Outline:

- Aims and Methods of the surveys
- Demographics of respondents
- Results overview: RWD usage, current issues, AI/ML usage, guidance use, interest in training, training topics

Roundtable

The roundtable session was moderated by Real4Reg partner Vera Ehrenstein (Aarhus University), with the following participants: Luca Giraldi (LG, European Medicines Agency), Niklas Hedberg (NH, Heads of HTA Agencies Group), Gianmario Candore (GC, Bayer), Carla Torre (CT, Faculty of Pharmacy of the University of Lisbon), and Paulo Gonçalves (PG, RD-Portugal).

Stakeholder perspectives on survey findings

LG noted that the high usage of Real-World Data (RWD) by Clinical Research Organizations (CROs) was expected. Also noted that the European Medicines Agency's

Pharmacovigilance Risk Assessment Committee (EMA-PRAC) relies heavily on RWD for safety evaluations.

NH expressed that he could relate with the survey data, but noted that there was a small number of responses from payers. Data quality and accessibility were the main focus of the survey; feasibility and methods could have received more attention.

GC emphasized the need for improved pre-study assessments of data quality, as data quality is the main challenge.

CT noted that while academic institutions conduct numerous studies, there is often limited interaction with regulatory agencies. This gap underscores the need for increased understanding of the regulatory landscape within academic research. She also pointed out the small number of respondents from academia and mentioned that while AI in academia is still a niche area, there is significant potential for growth. Agree with previous ideas, with data quality and access as key concerns, highlighting the importance of developing training programs that incorporate robust quality assessment processes.

Enhancing academic collaboration with other fields

GC emphasized that more than collaboration, we need communication. Need to share learnings and knowledges, successes and challenges, and also fostering transparency in how RWE is assessed.

NH highlighted that collaboration is the way forward and noted academia's key role in bridging the gap with other fields.

NH also emphasised that data quality alone cannot ensure reliable outcomes; trust in methodology and results is equally important. While clinical trials often rely on statistical significance, typically assessed by p-values, RWD lacks a unified standard for evaluation. Academia could be proactive in cross-disciplinary engagement. Academics have the opportunity to step forward and apply insights from diverse scientific fields, contributing new theories and methodologies to enhance RWD assessment practices.

Training strategies and stakeholder needs in real-world evidence

Real4Reg aims to develop training materials and has identified differences in training interests among stakeholders, raising the question of whether training should be joint with shared requirements or tailored to specific niches.

LG suggested that the first step is understanding the distinct training needs of each stakeholder group. As an example, EMA's training is designed to meet the specific needs of regulators.

GC proposed that transparency in training objectives could support a more collaborative approach.

NH suggested using examples from pilot projects in DARWIN EU as case studies during training sessions. These could expose stakeholders to varied questions and perspectives, helping identify "unknown unknowns."

CT agreed that transparency is essential and noted that while there is a common foundation in training, a fully unified program is challenging due to niche-specific needs. Surveys play a critical role in identifying and targeting these varied training needs.

Value of RWD

Vera questioned whether RWD might be better than commonly perceived. She noted that RWD is routinely collected and is not subject to researcher bias, and pointed out that, despite the terminology, studies using RWD still involve an intervention.

GC added that there are questions RCTs cannot answer, emphasizing the importance of clearly defining the question and determining the best source and methodology to address it.

NH raised a concern regarding patient selection, asking how we can verify that these are indeed the right patients. He acknowledged the uncertainty, stating that while we may not be sure if they are the appropriate patients, they are the patients we have.

Involving patients and patient organisations in training program development

PG emphasized that society needs to understand why RWD is interesting. It can be challenging to engage individuals who are unaware of the topic's importance. Thus, training should be conducted in plain language to ensure accessibility; without this, participation may be limited.

Without a clear explanation of the importance of RWD, data quality will not improve.

Patients should be involved at every stage of the process. Regarding survey response rates, when people understand the topic better, the number of responses increases.

Key Takeaways

- Key words: Communication, education, trust, involvement, and cooperation.
- There is a need to close the existing gaps between academia and regulatory agencies, as well as between academia and the general public. This can be achieved through joint training initiatives.
- Joint training sessions should be implemented to explore the different points of view of various stakeholders on the same topic and to identify what kinds of data are suitable for answering their specific questions.
- More transparency is crucial for gaining deeper insights into the activities of different stakeholders. This can help identify common ground among them.
- Incorporating use cases can help to understand different perspectives.