

## Real-World Data and Patients: Breaking the Ice

The Real4Reg consortium aims to ensure high visibility of the project and the promotion of active interaction, not only with specialists but also with patients who will ultimately benefit from the approval of new medicines. As stated in the Dissemination, Exploitation and Communication Plan, *“Apart from healthcare professionals, regulatory and health technology assessment (HTA) experts, industry, and payers, the patients and the general public are equally important stakeholders for (...) regulatory decision-making and HTA (...)”* and *“We will organise major symposiums within the RWD workshops aimed for patients and the general public in close cooperation with patients’ organisations so that attendance is maximised.”*

The second workshop took place in April 2026 and the details are provided below.

### Workshop Title: “Real-World Data and Patients: Breaking the Ice”

Date: 13 April 2026

Time: 15:00 - 16:30 CET

Online Platform: WebEx

### Agenda:

15:00-15:05	<b>Introduction</b>
15:05-15:35	<b>Real-World Data (RWD): Patient Perspectives and Applications in Healthcare</b> Presentation: Martin Russek (BfArM <sup>1</sup> ) Discussion: Margarida Oliveira (INFARMED, I.P. <sup>2</sup> ) and Dirk De Valck (EUALS <sup>3</sup> ) Moderator: Rebecca Moore (EIWH <sup>4</sup> )
15:35-16:05	<b>PatientsLikeMe: A Platform for RWD Sharing and Patients Engagement</b> Presentation: Eryn Macarayan (PatientsLikeMe) Discussion: Margarida Oliveira (INFARMED, I.P.) and Dirk De Valck (EUALS) Moderator: Rebecca Moore (EIWH)
16:05-16:25	<b>Q&amp;A</b>
16:25-16:30	<b>Closing</b>

<sup>1</sup> Federal Institute for Drugs and Medical Devices (Bundesinstitut für Arzneimittel und Medizinprodukte)

<sup>2</sup> National Authority of Medicines and Health Products, I.P.

<sup>3</sup> European Organisation for Professionals and People with ALS

<sup>4</sup> European Institute for Women’s Health

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

**Participants:**

The workshop had a total of 113 participants from 10 countries (Belgium, Denmark, Germany, Italy, Netherlands, Portugal, Spain, Switzerland, United Kingdom, and United States of America). A substantial portion of the audience was made by patients and patient organisation representatives (including presidents, directors of research engagement, board members, and patient services managers), however other stakeholder groups were also present, including: clinical research coordinators, pharmacists, nurses, project managers, researchers, regulatory affairs and pharmacovigilance specialists, professors, data scientists, and statisticians.

The audience also included members of the Real4Reg consortium and Advisory Board.

**Workshop Recording and Materials:**

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

**Summary of The Activities:**

**Presentation – Real-World Data (RWD): Patient Perspectives and Applications in Healthcare**

Speaker: Martin Russek (MR) (BfArM)

Outline:

- Patients Opinions and Perspectives on RWD Use Analysed in Real4Reg’s Survey Including Previous Knowledge, Future Importance, Data Sharing Concerns, and Willingness to Share Health-Related Data for Different Purposes
- RWD Applications in Healthcare, Namely in Academic Research, Private Sector Research, Regulatory Agencies, Education & Professional Training, and Public Health Monitoring

**Roundtable:**

The roundtable session was moderated by Rebecca Moore (EIWH), with Dirk De Valck (DV)(EUALS) and Eryn Macarayan (EM)(PatientsLikeMe) as participants.

*Patients Organisations Initiatives to Increase Patient’s Trust in Sharing Their RWD*

From DV’s experience and close connection with patients, privacy is a common concern. However, specifically for amyotrophic lateral sclerosis (ALS) patients, this is usually less significant than in people with other conditions, since there is currently no effective drug to treat ALS. Therefore, ALS patients are usually very open and willing to share their data and to participate in clinical trials. DV believes that sharing patients’ data in an unidentifiable manner could improve trust and one solution might be to use it as synthetic data.

*Patients RWD Importance and Usefulness for Regulatory/HTA Agencies*

MO believes that the use of RWD and real-world evidence (RWE) will certainly increase in the future, to support regulatory decision-making, HTA decisions, and marketing authorisations. MO also mentioned that INFARMED, I.P. has been incorporating patients input in HTA decisions, with a very positive impact in these stakeholders. For MO, RWD is extremely important to regulatory and HTA bodies, since clinical trials populations are not representative of the people that will actually use the medicine in real practice. Therefore, this is the only way to assess the new technology real impact in the community.

Ways to Encourage Patient's Involvement in Decision-Making

DV said that EUpALS is in close contact with European's Medicines Agency (EMA) and companies that set up clinical trials. Their work as a patient organisation is to proofread the documents related to the clinical trials, including protocols, informed consents, and patient brochures, and provide feedback on how understandable they are for the average patient. DV mentioned that providing this information to the patients in a comprehensible manner helps to increase their willingness to participate because they can clearly understand the procedures. In addition, DV also suggested that in the future the use of a validated AI system that can be applied to a validated dataset will facilitate approvals and concordance between EMA and HTA bodies decisions.

MO believes to be important the improvement of the communication between regulatory/HTA agencies and patients, since the language used in these fields can be very technical and include some acronyms that might not be understood by patients. In addition, MO also mentioned that is really important for these bodies to completely describe their activities and specify where and when the patient input is truly needed. The medicines approved at European level are highly innovative and therefore patients' involvement is crucial. In MO point of view, patient's data sharing is a way to support the approval of a medicine for a different indication or an extension of its use, ultimately benefiting patients.

**Presentation – PatientsLikeMe: A Platform for RWD Sharing and Patients Engagement**

Speaker: Eryn Macarayan (EM) (PatientsLikeMe)

Outline:

- Main Goals and Vision
- Functionalities and Ways to Use (e.g. RWD sharing, forums with other patients, AI personal health navigator tool...)
- Types of RWD Shared (e.g. diseases/conditions, symptoms, treatment and side effects, quality of life...)
- Informed Consent and Privacy Aspects

- Patient Reported Benefits (e.g. improved symptom management, better understanding of important factors for treatment decision-making, better conversations with healthcare professionals...)
- Clinical Trials and Research Studies Opportunities

#### Relevance and Importance of Online Patient Communities

DV mentioned that PatientsLikeMe was one of the first platforms where patients could share their data, allowing similar projects to arise. He said that this platform was really accessible given the fact that not only it has a website, but also an app that patients can use. One of the main advantages of online patient communities is that patients can give their contribution whenever it is convenient for them throughout their day.

#### Usefulness of the Data Shared in Online Patient Communities to Regulatory Decision-Making and HTA

MO mentioned that platforms such as PatientsLikeMe can be really helpful in gathering patients' data and providing it in a straightforward manner. MO stated that even though almost all regulatory and HTA decisions are currently made based on clinical trials' information, these do not reflect for example how treatments actually occur in real life, how doctors deal with the different options available or how patients adhere to the treatment regimens. In addition, MO also said that these data are extremely important from populations that are excluded from clinical trials, to really understand how the outcomes from clinical trials will actually translate on the clinical practice and for the assessment of the medicine's lifecycle in HTA bodies.

#### Future of Using Patient-Driven Data Sharing Platforms

DV mentioned that patient-driven data sharing initiatives are becoming more common and in his perspective in the future these could merge into a one wide world platform where Food and Drug Administration (FDA), EMA, and HTA bodies could retrieve information from.

MO emphasised that patient-driven data sharing platforms can be a way of ensuring that data is owned by patients and that they are included in decision-making, instead of being

just a way of getting the information needed. In addition, MO also believes that these initiatives can ensure the data is representative of specific populations.

### **Questions**

**Question for EM:** *“Do you see some kind of merging and acquisition in the data sharing platforms field?”*

EM believes that this scenario is a possibility and mentioned that as data are becoming more aggregated, especially with AI, this gathering has to have a purpose and a high impact to the patient in particular.

**Question for EM:** *“Can only patients see what each other are sharing or also outside viewers can have a look?”*

EM stated that PatientsLikeMe puts the level of consent and participation on the patients' side. Therefore, they can choose whether the data they are sharing is for public view or if they prefer to keep it private. EM believes that having this option translates into a better patient experience, since it is up to the individuals to define what kind of consent to give in regards to how their data is used.

**Question for MR:** *“What about convincing physicians to talk to and help their patients to share data as from Martin’s presentation it seems that they are more reluctant and do not often inform their patients about the advantages of RWD?”*

MR mentioned that physicians are in fact slightly more hesitant in RWD sharing than the patients themselves. On one hand, he believes that there is a need to educate physicians on how data is used and which data-sharing platforms are safe. On the other hand, MR mentioned that even though physicians do not have unlimited time with each patient, it would be important to explain and convince them that it is useful to talk to patients or their caregivers in order to encourage them to share health-related data.

EM added that there is a huge physician burden that needs to be addressed, however explaining to them how to really use the data would be crucial.

**Question for EM:** *“How do you address the historical issues with PatientsLikeMe and that the current governance is outside of HIPAA compliance. This is a social platform and not a medical platform, correct?”*

EM said that PatientsLikeMe is a social platform where all information corresponds to self-reported data. Therefore, for specific use cases the website is currently developing private environments called ‘sponsored communities’, where patients are required to validate their self-reported data, for example through the submission of medical records. Regarding Health Insurance Portability and Accountability Act (HIPAA), the PatientsLikeMe platform is currently not under it, however the data are treated accordingly to HIPAA’s strict rules and principles, especially when they are part of a research or an ongoing specific engagement.

**Question for EM:** *“Are you also making specific calls for patients with specific diseases to ensure collecting a meaningful amount of specific data?”*

EM shared that PatientsLikeMe currently has almost 8000 conditions and not all of them have had a one-on-one engagement. However, for specific use cases the platform has nurse coordinators that patients can interact with and whose role include the information’s collection and the validation of the self-reported data. In addition, she expressed her hope of having this functionality available for all conditions included in the platform.

**Question for EM:** *“PatientsLikeMe seems to be a perfect, holistic and engaging digital platform. Is it based in United States (US)? Follows GDPR of US? How do you guarantee the sustainability? Only available in English?”*

EM mentioned that the platform is primarily US focused, however it is available and accessible from the European Union as well, currently counting with some memberships. To guarantee data privacy and security, PatientsLikeMe has a compliance team that reviews General Data Protection Regulation (GDPR) policies and guidelines and ensures that when working with the data a compliance process is followed.

**Question for EM:** *“How representative is PatientsLikeMe data considered to be? Does it span wide age/ethnicity/socio demographic groups? And what is your experience of how long patients stay engaged with PatientsLikeMe beyond their first enthusiasm for the platform?”*

EM mentioned that the majority of the platform users were women, which encouraged the creation of a new specific website – WomenLikeMe – where women can talk to each other and share their experiences. Regarding ethnicity, EM said that the platform is open and free to all, which helped in increasing the ethnical variations and ensuring that each one is represented. In particular researches, specific efforts are made to ensure ethnicity representativeness, for example translations in different languages and interaction with patient advocates to spread the word about the work being developed and encourage patients who are less inclined to take part (e.g. due to the lack of digital health literacy).

**Question for MR:** *“Which would you say are the main advantages and disadvantages of RWD use in comparison with clinical trials?”*

MR said that RWD has two main advantages, namely size representative results (especially important in rare diseases context) and reduced research burden for everyone involved, particularly patients and physicians. In terms of disadvantages, MR mentioned that from a statistical point of view, the data can only be observed and no intervention can be made, for example randomly assigning treatments to patients, and therefore there is a need to have a methodology and assumptions to ensure that two treatments are being compared on a fair basis, instead of on any kind of pre-determined probability or confounding effect.

DV added that he believes we are currently in the beginning of the AI era, which will certainly help patients. From DV’s perspective, when the right algorithms emerge, they will lead us to the digital twins concept which would benefit patients due to the fact that the placebo can be placed on the data and patients can be absolutely sure that they receive the medicine that is being studied in the clinical trial.

MO mentioned that currently clinical trials provide the most supportive data for regulatory and HTA decisions, however RWD is bringing complementary information that is very needed and useful for decision-making.

EM agreed with DV’s perspective on RWD being able to provide an historical wealth of information that could help patients join clinical trials. In addition, EM mentioned that clinical trials have very specific criteria and endpoints, while RWD can provide an overall picture of the outcomes. From EM’s perspective, even though clinical trials have a lot value, RWD shows the evidence lived by the patients, which is what truly matters and, therefore, these two should be combined.

**Key Takeaways:**

- Key words: data privacy/security, representativeness, collaboration, patient involvement, accessibility, informed consent, data-sharing platforms, decision-making.
- Patients' main concerns when sharing their RWD include data privacy/security, ethics issues, and perception of lack of benefits. These can be demystified through a clear communication with experts from regulatory and HTA bodies and also with physicians, given their close relationship with patients.
- Patient involvement, through RWD sharing, is crucial for regulatory and HTA decisions, since the information provided by clinical trials is limited, both in follow up time and in the population represented. Therefore, RWD can demonstrate how a new medicine or health technology actually impacts patients in real life.
- Even though the majority of patients are willing to share their RWD (especially people with rare and/or high mortality rate diseases, for example ALS) for activities such as research or monitoring, they report a lack of knowledge and education on RWD.
- Patients' organisations make the connection between patients and specialists by translating into plain language the initiatives developed (e.g. clinical trials) and decisions taken, ensuring these stakeholders understand every step which in turn will encourage them to actively participate.
- Patient-driven data sharing platforms can be helpful to manage symptoms, adjust to a new condition, and improve conversations with healthcare professionals. These can also ensure that the data is owned by patients, giving them the confidence to participate in decision-making and helping them feel more seen and understood.

## Appendix – Comments From the Chat

- MRB - Good afternoon! Welcome to Real4Reg's Patients Workshop. Please use the chat feature to ask questions or provide comments. If you prefer, during Q&A you can raise your hand and ask your question directly to the speakers or discussion participants.
- R – I'm muted
- R - I can't unmute myself
- NS - Congratulations on the initiative for this excellent webinar.
- NS - My question relates to the ethical validation of studies that use real-world data. Is this issue being discussed? Are there specific parameters that should be considered, beyond the already identified issue of privacy? Are there ethics committees specialized in this type of study? Would an opinion from an ethics committee not serve as a safeguard to ensure patient beneficence and autonomy?
- V - What about convincing physicians to talk to and help their patients to share data as from Martin's presentation it seems they are more reluctant and do not often inform their patients about the advantages of RWD?
- SB - How do you address the historical issues with PLM and that the current governance is outside of HIPAA compliance. This is a social platform and not a medical platform, correct? Do you see that inclusion of sensitive medical data will be respected and speak the location of the data, who, when, and what data had been accessed and by whom. A primary legal issue concerns the monetization of sensitive patient data. Can you confirm if PLM is compliant to EU data laws if data is provided by a EU citizen, if data is not held within the European data space.
- V - To Erlyn. For PML, sorry if I missed it; are you also making specific calls for patients with specific diseases to ensure collecting a meaningful amount of specific data?
- EM - Yes we do. We have call coordinators for specific use cases.
- TM - Erlyn, PLM seems to be a perfect, holistic and engaging digital platform. Is it based in US? Follows GDPR of US? How do you guarantee the sustainability? Only available in English?
- AG - How representative is PLM data considered to be? Does it span wide age / ethnicity / socio demographic groups? And what is your experience of how long patients stay engaged with PLM beyond their first enthusiasm for the platform?
- E - Thank you for this initiative. Very interesting 😊
- TM - Thank you
- JLS - Thank you for the session!
- NS - 👍
- V - thank you
- JR - Thank you!
- LM - Thank you for the session