RWE4Decisions REAL WORLD EVIDENCE

EVENT REPORT

Towards Establishing an EU Real-World Evidence Generation Action Plan for Better Healthcare Systems & Patient Outcomes

24 November 2021

SPEAKERS

Moderated by Jacki Davis

KEYNOTES

Frank Vandenbroucke
Belgian Deputy Prime Minister and Minister for Social Affairs and Public Health

Rui Santos Ivo
President, National Authority of Medicines and Health Products (INFARMED)

PANEL 1: RWE4DECSIONS LEARNINGS – REAL-WORLD EVIDENCE IN OUTCOMES-BASED MANAGED ENTRY AGREEMENTS

Karen Facey
Visiting Senior Research Fellow, Usher Institute, University of Edinburgh

Nicole Mittmann
Chief Scientist and Vice-President of Evidence Standards, Canadian Agency for Drugs and Technologies in Health (CADTH)

Hervé Nabarette
Deputy Director for Public Affairs, AFM-Telethon

Anna Nachtnebel
Senior HTA Expert, Austrian Social Insurances

Karen Coulton
Global Head of Payer Engagement, AstraZeneca

PANEL 2: REAL-WORLD EVIDENCE FRAMEWORKS IN CROSS BORDER COLLABORATION

Aldo Golja
Senior Policy Advisor on Drug Pricing and Reimbursement, Ministry of Health, Welfare and Sports, The Netherlands

Flemming Sonne
CEO, Amgros, Denmark

Tuomas Oravilahti
Pharmacoeconomist, Finnish Medicines Agency (FIMEA), Finland

PANEL 3: BUILDING BLOCKS FOR ESTABLISHING AN EU REAL-WORLD EVIDENCE GENERATION ACTION PLAN

Peter Arlett
Head of Data Analytics and Methods Taskforce, European Medicines Agency (EMA)

Alexander Natz
Secretary-General, European Confederation of Pharmaceutical Entrepreneurs

Judith Fernandez
Coordinator – RWE unit, HTA division, Haute Authorité de Santé (HAS)

Yann Le Cam
Chief Executive Officer, EURORDIS – Rare Diseases Europe

Flora Giorgio
Deputy Head of Unit, Unit for Medical Devices and Health Technology Assessment, DG SANTE, European Commission

CONCLUDING REMARKS

Jo De Cock
Former CEO, Belgian National Institute for Health and Disability Insurance (INAMI/RIZIV)

Keynote speeches

Frank Vandenbroucke
Belgian Deputy Prime Minister and Minister for Social Affairs and Public Health

- The principles of scientific evidence and solidarity are particularly important for policymaking. We need real world evidence (RWE) for our decisions on reimbursement, thus ultimately contributing to the financial accessibility of medicines in a spirit of solidarity.
- Evidence is crucial to make informed decisions, and the digital revolution offers enormous opportunities for healthcare. However, its exploitation takes effort and requires new investments.
- With the establishment of the Belgian Health Data Authority, which will be linked to the European Health Data Space, Belgium is taking an important step in developing a 'registration and evaluation system' to enable the generation of real-world data (RWD) on a national scale. This authority will ensure that all conditions are met to make data available for all aspects of health, such as population management, R&D, policy information, financing of healthcare, etc. Federal administrations are already in advanced consultations, which will also be expanded to other stakeholders, including healthcare providers and healthcare institutions, the industry, Europe, and the citizens themselves.
- There is no doubt that there is work to be done. (…) Challenges still remain in terms of data available vs data needed, delay in the availability of data, questions of cooperation of clinicians and patients, questions of trust… For a number of challenges an international approach at European level can offer a perspective.
- The RWE4Decisions initiative, launched by the Belgian Reimbursement Authority (INAMI-RIZIV) five years ago, has already delivered important results and launched good debates in an international and multi-stakeholder setting. It has also shown that a better interaction between regulators on European level and payers on national level must be achieved so that payers' questions are also sufficiently addressed in an evidence generation plan.
- Ideally, it should be possible to come to an evidence generation plan that provides transparent data that responds to the needs of both: the regulators' needs regarding safety and efficacy, as well as the payers' needs regarding clinical and cost-effectiveness.
- The use of RWD for decision-making is not only a technical topic, essentially, it is a political debate about the correct allocation of scarce resources, and about ethics.

The Minister's full speech is available here.

Rui Santos Ivo
President,
National Authority of
Medicines and Health
Products (INFARMED)

- RWE can complement regulatory knowledge, reduce evidence gaps in HTA and Payer decisions for highly innovative therapies and support medical decisions on best treatment options.
- The focus should be on the common needs of regulators, HTA and Payers and to foster synergies.
- The Council Conclusions adopted during the Portuguese EU Presidency state that improved data requirements may benefit from dialogue between regulators, HTA bodies, Payers, patients and healthcare professionals.
- Member States and the European Commission are also invited to establish a RWE data collection and evidence generation action plan by promoting better collaboration between ongoing national and cross-border initiatives – such as the development of a robust framework and methodology via a multi-stakeholder approach.
- Stakeholders will need to collaborate in accessing and analysing RWD, EU initiatives like DARWIN EU, the European Health Data Space will play an important role to facilitate this.
- The revision of the general pharmaceuticals legislation provides a unique opportunity to adopt an integrated framework which addresses the needs of the different users of RWE.
- A high-level platform on RWE at EU-level could be a concrete starting point to ensure a coordinated agenda on issues such as: the availability of health data, common data elements and formats, quality assurance, data standards, common policies to minimise barriers for data sharing, health system management and research.

Panel 1 : RWE4DECSIONS LEARNINGS – REAL-WORLD EVIDENCE IN OUTCOMES-BASED MANAGED ENTRY AGREEMENTS

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RWE4Decisions hosted in June 2021 two case study workshops with the objective to agree on a real-world evidence framework in Outcomes-Based Managed Entry Agreement (OBMEA) for two fictitious cases: One of the cases was for a therapy given at regular intervals to children with a rare neuromuscular disease, while the other one was for a one-off cell therapy given to adults in a late-stage cancer. The workshops focused on identifying the decision-relevant uncertainties in these two cases. During this panel, Karen Facey presented the recommended actions to support the successful implementation of OBMEAs.

In response to the actions, panellists raised issues relating to:

- Requirements for public documentation of OBMEAs given confidential price & reimbursement processes
- The role of industry funding in OBMEAs
- The need to advance data infrastructures
- The meaningful engagement of patients throughout the OBMEA process
- The value of international collaboration and exchange of best practices

A link to the actions can be found on the RWE4Decisions website here:
https://rwe4decisions.com/documents/

Panel 2 : REAL-WORLD EVIDENCE FRAMEWORKS IN CROSS-BORDER COLLABORATION

Beneluxa

Aldo Golja
Senior Policy Advisor on Drug Pricing and Reimbursement,
Ministry of Health, Welfare and Sports,
The Netherlands

- Beneluxa is a voluntary collaboration (Belgium, Netherlands, Luxembourg, Austria and Ireland), which collaborates in the International Horizon Scanning Initiative, provides joint collaboration on health technology assessments (HTA), and jointly negotiates prices (most recently on Zolgensma).
- Beneluxa recognises value in collaboration with regards to the use of real-world evidence. It has experience in defining common criteria for required additional data in HTA assessments; It has actively engaged with manufacturers and clinicians to define relevant outcomes to assist in joint HTA work; it also sees the opportunity to explore consensus on useful outcomes with other payers and HTA bodies; it can be beneficial in sharing resources and expertise.
- However, Beneluxa doesn't currently have use cases with RWD. Post-assessment data collection is easier said than done.
- Cross-country collaborations can serve as a testing ground for RWD approaches, and Beneluxa is interested in pursuing that further, in collaboration with other stakeholders and initiatives.

Nordic Pharmaceutical Forum

Flemming Sonne
CEO, Amgros,
Denmark
and Chairman of Nordic
Pharmaceutical Forum

- The Nordic Pharmaceutical Forum (NLF) is a voluntary collaboration set up in 2015, as an informal space to identify opportunities, knowledge sharing and work towards common Nordic solutions. It currently involves Iceland, Norway, Sweden and Denmark.
- NLF is working to identify a Nordic Framework to deal with a high clinical uncertain data pool when accessing outcomes-based managed entry agreements, especially in its work on new expensive drugs and in the area of horizon scanning.
- When it comes to the collection and use of RWD, the EU is better placed than national level/collaborations to identify the common opportunities and define the place for real-world data.
- Collaboration between countries can help to ensure post-launch follow-up on the drug.

FINOSE

Tuomas Oravilahti
Pharmacoeconomist,
Finnish Medicines Agency
(FIMEA), Finland

- FINOSE is a regional collaboration between Finnish (Fimea), Norwegian (NoMA) and Swedish (TLV) HTA bodies, which was launched in 2018. FINOSE collaborates on clinical assessment and economic evaluations but does not engage in joint price negotiations.
- The FINOSE collaboration engages in joint work during normal processes: company submissions are submitted at the same time, and a FINOSE report is published (with some local details added as needed). In practice, three decisions are made (Fimea/NoMA/TLV decision), but sometimes joint negotiations are possible.

Panel 3 : BUILDING BLOCKS FOR ESTABLISHING AN EU REAL-WORLD EVIDENCE GENERATION ACTION PLAN

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Head of Data Analytics and Methods Taskforce, European Medicines Agency (EMA)

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Deputy Head of Unit, Unit for Medical Devices and Health Technology Assessment, DG SANTE, European Commission

- Real-world evidence (RWE) is a complementary - and not a competing - source of evidence for decision-makers along the life-cycle of products. In rare diseases, RWE has majorly contributed to primary demonstrations of efficacy.
- Sustainable funding models, political support and legislation are needed to enable the use of RWE for decision-making.
- A proportional approach is crucial – meaningful and useful questions for medical practices are more important than focusing on capturing all data.
- Solutions cannot be limited to a national or regional level but must include EU-level guidance and infrastructures.
- An EU fund for the collection of RWE post-launch could improve access to innovative therapies in Member States.
- Patient involvement is important to ensure right questions are being asked.
- The European Medicines Agency has a clear vision for enabling use and establish value of RWE by 2025.

"In facing common challenges, let's do it together and in trust"

Jo De Cock, former CEO, Belgian National Institute for Health and Disability Insurance (INAMI/RIZIV)

The RWE4Decisions multi-stakeholder work in 2021 concluded:

1. RWE is not a panacea or a stand-alone issue - however **RWE has a clear place within, and can be a vital part of, an integrated evidence generation plan for highly innovative technologies.**

2. **Ongoing and structured light touch iterative dialogues** among stakeholders **along the life-cycle of a product** should be developed further. These interactions are needed to identify the specific gaps and uncertainties with regard to access, availability and affordability of highly innovative technologies that could potentially be resolved by RWE generation.

3. Integrated evidence generation plans should address the needs of regulators, HTA and payers. There is a need to move from **«fragmented recommendations to comprehensive guidance» to improve the standards and the credibility of RWD studies.**

4. **To support outcomes-based managed entry agreements (OBMEAs)** that can inform optimal use of high-cost therapies, **there is a need for a clear RWE generation plan**, that takes account of regulatory post marketing data collection requirements**.** This requires **financial investment in data infrastructure and alignment of approaches across borders.** DARWIN EU and the European Health Data Space will have a crucial role to play in this regard.

5. **All stakeholders** must contribute to the OBMEA to **deliver high quality real-life clinical data** that could reduce the decision-relevant uncertainties identified at the point of pricing and reimbursement. The RWE collected in OBMEA also has an important role in treatment optimisation.

6. All these elements prove that there is a need to support decision-makers through the **sharing of practical learnings** on the use of RWE in HTA/Payer decisions. RWE4Decisions will continue to work collaboratively with stakeholders, the European Reference Networks and other initiatives to develop clearer processes and guidance that identifies how robust RWE can be developed for HTA/Payer decisions.

A Steering Group will be established to guide the work plan of RWE4Decisions 2022 and determine the sustainability of this agenda.

RWE4Decisions REAL WORLD EVIDENCE

RWE4Decisions is a multi-stakeholder group, which has developed stakeholder actions that will better enable the use of real-world evidence in HTA/payer decisions about highly innovative technologies. The work has been commissioned by the Belgian National Institute of Health and Disability Insurance (INAMI-RIZIV) and contributors include HTA bodies and payers, regulatory agencies, patient groups, clinicians, industry and academic experts/researchers.

For further information and to watch the recording of the Conference, visit our website at https://rwe4decisions.com/event/towards-establishing-an-eu-real-world-evidence-generation-action-plan-for-better-healthcare-systems-patient-outcomes/

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