RWE4Decisions REAL WORLD EVIDENCE

Webinar Report

Beating Cancer with Highly Innovative Technologies - What's the Role of RWE?

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Co-moderated by

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European Cancer Organisation Past President & Medical Oncologist

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Speakers

Christine Chomienne
Professor of Cellular Biology *Université de Paris*; Vice-Chair, *EU Cancer Mission*

Tomislav Sokol, MEP
ENVI Co-Rapporteur for the European Health Data Space, *European Parliament*

Tanja Podkonjak
Director, EUCAN Oncology Access & Reimbursement Policy, *Takeda*

Natacha Bolaños
Head, Membership & Alliances, *Lymphoma Coalition*

Emma Hernlund
Analyst, *Swedish Dental and Pharmaceutical Benefits Agency (TLV)*

The RWE4Decisions webinar series in 2023 focuses on the products that will be the first to undergo Joint Clinical Assessment under the new Health Technology Assessment (HTA) Regulation: cancer medicines and Advanced Therapeutic Medicinal Products (ATMPs). As there is often a paucity of clinical evidence available for these innovative treatments, the potential for use of Real-World Evidence (RWE) must be explored, taking into consideration the existing challenges, such as the lack of EU-wide guidance on RWE generation or use.

Cancer is a societal issue, as 2.7 million Europeans are diagnosed yearly, and other 1.3 million succumb. As these numbers are set to increase, the European Union has implemented large-scale public policies to "beat cancer". **Professor Christine Chomienne** presented an overview of the **EU Mission on Cancer** that was launched in September 2019 to save more than 3 million lives by 2030. The Mission on Cancer has formulated 13 recommendations, many of which refer to the use of Real-World Data (RWD), to ensure patients live longer and better.

Professor Chomienne stressed that **to drive cancer research forward, all available data must be utilised in a wasteless and coherent mindset**, because they bring added value to every stakeholder and Member State (MS). Patients support the need to share data (with appropriate governance measures) to support knowledge development, avoid waste and overcome fragmentation of systems - developing legitimacy and building trust.

The **UNCAN.EU** federated data hub is a flagship initiative of the EU Mission on Cancer that is being developed to **enable researchers from all over the world to share and access high quality data** from a range of sources. It will be managed by Member States, associated countries, and stakeholders.

The future **European Cancer Patient Digital Centre** will be a national portal for patients to access, deposit and exchange their data. The primary purpose is to create a health passport and knowledge centre for individual patients to foster a **long-term personalised care plan**. The secondary purpose is to provide **interoperable data** that can be shared (if a patient chooses) for research and policy purposes.

Professor Chomienne concluded that **equity of cancer care requires cross-border collaboration among MS, using all available data from all stakeholders**. This requires trusted frameworks for data access and the demonstration of added value for each stakeholder. Importantly for RWE4Decisions, she noted that **not only do RWD need to be fit for the research or policy question, but research questions needed to be adapted to the available data.** Furthermore, the robustness of evidence that is needed for decision-making must be clarified.

Following Professor Chomienne's keynote presentation, the panellists representing various fields shared their views on the role of RWE to support HTA/Payer decision-making about cancer treatments.

MEP Tomislav Sokol gave an update on the legislation to support the **European Health Data Space (EHDS)**, which is a unique opportunity to support primary use and appropriate secondary use of health data across the EU for research and policy purposes. Secondary use would only be permitted for bona fide purposes approved by a health data access body in the relevant MS. To ensure patient protection, privacy, and safety, only anonymised or pseudonymised data would be accessible within a Secure Operating Environment. **Stakeholders from universities, research institutes and industries would then be able to collaborate and use secondary data sets to develop innovations and improve the quality of life of patients**. Patients with rare diseases, including cancer, will potentially benefit the most, as will the smaller MS. MEP Sokol also shared the obstacles the legislative file is currently facing, including the negotiations surrounding an opt-out mechanism for patients sharing their data, **mis-information about the purposes for which data might be used which is damaging public trust**, the need for additional funding to be able to work with the data infrastructures in each MS, education of healthcare professionals and citizens, as well as the ambitious timeline for adoption.

Tanja Podkonjak presented an industry viewpoint and stressed the ever-growing evolution of advanced and precision therapies in oncology, most particularly in the field of rare cancers. For many of these medicines, **questions cannot be answered by standard clinical studies, especially when patient populations are small or when there are large variations in standards of care**. RWD can consequently supplement Randomised Clinical Trials (RCTs), only if safeguards are implemented to ensure rigor and quality. **Development of robust RWE is complex and costly and thus clear guidance, such as NICE's 2022 RWE framework is needed**. There is hope that the EHDS will help create a harmonized and safe framework for data access that will foster multi-stakeholder collaboration, which is essential to ensure that the lives of cancer patients are improved.

Representing the patients' perspective, **Natacha Bolaños** stressed that COVID-19 accelerated the need to efficiently collect and share data amongst MS, and that we need to learn from that experience. **RCTs do not report real life outcomes nor provide a clear understanding of the reality of the disease and the complexity of care** in the real world. This is particularly true in relation to co-morbidities and usual care. Hence RWD plays a pivotal role, particularly quality of life information – both quantitative and qualitative. **Patient groups want to contribute meaningfully to partnerships that support RWD collection, and ensure that RWD contributes to decision-making.** EU patient organisations are investing resources to collect RWD, particularly in relation to unmet needs and want to see this driving development of new medicines. Further to MEP Sokol's intervention, Natacha highlighted the need to

understand why citizens don't want to share data, so that answers can be provided, and trust built.

Emma Hernlund shared insights from an HTA body perspective. A wide range of evidence informs an HTA, with long-standing use of RWD to build economic models to determine cost effectiveness. Comparative effectiveness has traditionally been demonstrated by RCTs. However, **as many innovative cancer medicines are being submitted to HTA bodies with single-arm trials, comparative effectiveness must rely on indirect comparisons, many of which use RWE**. This puts higher demands on the transparency, planning, execution, and analysis of the RWD. However, **HTA bodies recognise that there is a lack of harmonized guidance about how to critically assess quality of RWD and robustness of RWE**. Collaboration and code-sharing are needed, by health technology developers to HTA bodies and amongst HTA bodies.

The following questions raised by the audience were discussed with the panellists:

- How can we foster partnerships within stakeholder groups across MS, and among stakeholders, to collect better RWD?
- How can we support routine collection of Patient Reported Outcomes (PRO), patient experience and patients' preferences data?
- Considering the cost of developing RWE, how could industry be incentivised to invest in the field of rare diseases?

In conclusion, panellists agreed that **all stakeholder groups must collaborate to build trust in appropriate secondary use of health data** for research and policy purposes and ensuring strict privacy safeguards. With the changes to the European health landscape induced by both the EHDS and the HTA Regulation, **RWE should play an increasingly important role in the assessment of new treatments for cancer**. The effective use of RWD in the fight to beat cancer requires building capacities, work in partnerships and enhance transparency of methods, both in terms of RWE generation, and its critical assessment.

RWE4Decisions is a payer-led multi-stakeholder learning network, 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 is led by a multi-stakeholder Steering Group with a wider community of contributors including HTA bodies and payers, regulatory agencies, patient groups, clinicians, industry, analytics experts and academic experts/researchers.

For further information and to watch the recording of the webinar, visit our website at:

https://rwe4decisions.com/event/beating-cancer-with-highly-innovative-technologies-whats-the-role-of-rwe/

What are you are doing to progress learnings on the use of RWE?

Contact us at **secretariat@rwe4decisions.com** to join the RWE4Decisions Learning Network.

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