

REAL WORLD EVIDENCE
RWE4Decisions
EVENT REPORT

Delivering a Health Data Strategy for the European Reference Networks: Developing a Shared Vision with All Stakeholders

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In September 2021, RWE4Decisions held a webinar to reflect on the need for a long-term vision and a holistic data strategy for the European Reference Networks (ERNs).

The ERNs are a major infrastructure for care delivery and research and offer great potential for the innovative use of health data across borders. For rare diseases, no country has enough data, so we need EU collaboration. Established by the European Commission in 2017, there is now an opportunity for the ERNs to define their ambition for the next five years. The conclusion of this RWE4Decisions discussion was that there is a need for an ERN data strategy, defined through multi-stakeholder collaboration, and involving the HTA authorities and payers so the ERNs can contribute to HTA/Payer data needs and support the collection of real-world evidence (RWE) in assessing the value of highly innovative technologies

The proposed solutions should inform the European Commission evaluation of ERNs (foreseen in 2022) as well as the current evaluation of the Cross-border Healthcare Directive (establishing the legal basis of ERNs.)

Key Learnings

Challenges to be addressed

- **Lack of (semantic, technical) interoperability** - Fundamental problems around improving interoperability include standardization of terminology and normalization of data to those standards. The high volume of data healthcare IT systems produce exacerbates these problems.
- **Poor quality data** – If the data is incomplete, inaccurate, inconsistent or not updated, patients cannot trust doctors/hospitals to make the best decisions for them. Data of good quality is essential for example in order to implement performance-based risk-sharing agreements.
- **Legal barriers and differing regulatory obligations** - The introduction of the General Data Protection Regulation (GDPR) in 2018 has helped to protect individual's rights, but has been more difficult for the secondary use of data because interpretation or implementation of GDPR diverge across different national contexts, propagating uncertainty for the global pharmaceutical industry and further hampering opportunities for research (see [EC report 2021](#)).
- **Lack of funding** – National funding for ERN-related services and activities is still lacking in the majority of Member States, including innovative service delivery models (usage of Clinical Patient Management System (CPMS)), networking and administrative activities, or maintenance of infrastructures (e.g. collection of data for ERN registries). EU funding currently does not ensure long-term sustainability for many long-term activities of ERNs.

Solutions / Possible Way Forward

A Data Strategy for ERNs should :

- 1. be supported by EU funding.** EU4Health is a new financial instrument (2021-27) with significantly higher budget than previous years. **Registries** are a necessity for research, epidemiology, data trials. There are currently more than 30 ERN registries. Some registries are already functioning, others are getting build.
- 2. work on ensuring registries have the data sufficiently structured and standardized so the data is FAIR** (Findable, Accessible, Interoperable and Reusable). Data are in the hospitals, often in an unstandardized and unstructured way. It is crucial for ERN registries to harmonize the data collection as much as possible by using standards ontologies. Interoperability of the databases is key so that data can be searched across the different registries.
- 3. be driven by ERNs' data needs** and at the same time well aligned with the wider policy agenda, including the European Health Data Space and the work of the European Joint Programme on Rare Diseases (EJP RD). To inform decisions, HTA bodies need data - the EHDS has great potential to deliver for the HTA community and HTA/Payers' network.
- 4. be anchored at national level.** All the centralized structures will not work unless they have a formal contribution from the local sides. First rare disease patients need to be identified with the help of Orphanet and the European Commission at the local/site level. Without the local support, data collection will be slow and inefficient. Once the data is identified at the source, it needs to be made available through the EHDS and registries.
- 5. reach countries that are most in need.** Tools such as registries and CPMS (virtual clinic to resolve difficult cases of Rare Diseases) should be implemented in central and Eastern Europe. The tools have to be user friendly for clinicians and patients, engaging professionals/HCPs.
- 6. be inclusive of all stakeholders. A multi-stakeholder partnership** including industry could be set up to create a comprehensive and holistic data strategy for ERNs and build trust. Multi-stakeholder discussions should also involve HTA, Payers and regulators.

Ongoing initiatives

European Joint Programme on Rare Diseases (EJP RD)

Virtual platform and one-stop shop to discover, query and eventually access a variety of data sources (e.g., patient registries). It brings together over 130 institutions (including all 24 ERNs) from 35 countries.

European Rare Disease Research Coordination and Support Action (ERICA)

Focus on harmonized data capture, protection, and access across ERNs. Development and integration of ERN-wide rare disease registries and their utilization for joint research

Joint Action Towards the European Health Data Space (TEHDAS)

Develops European principles for the secondary use of health data. TEHDAS started in February 2021 and is being carried out by 25 European countries and co-ordinated by the Finnish Innovation Fund, Sitra.

European Commission Joint Action on Integration

Will support Member States in developing concrete proposals to make ERNs an integral part of their national healthcare systems



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 webinar, visit our website at <https://rwe4decisions.com/event/delivering-a-health-data-strategy-for-the-european-reference-networks/>

Do also follow us on [Twitter](#) and on [LinkedIn](#)

We want to hear what you are doing to progress learnings on the use of RWE! Contact us at secretariat@rwe4decisions.com if you would like us to include any relevant updates to our next quarterly newsletter coming out in November.