RWE4 Decisions project: how to take more informed reimbursement decisions with RWE?

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March 12th 2021

RWE – RIZIV-INAMI papers

2016	2017	2018	2019/2021
The use of real world data throughout an innovative medicine's lifecycle	Outcomes based pricing and reimbursement of innovative medicines with budgetary limitations	TRUST4RD – Tool for Reducing Uncertainties in the evidence generation for Specialised Treatments for Rare Diseases	Real-world evidence to support Payer/HTA decisions about highly innovative technologies in the EU – actions for stakeholders
INAMI-RIZIV	INAMI-RIZIV	orphanet TRUST4RD Tool for Reducing Uncertainties in the evidence generation for Specialised Treatments for Rare Diseases	Official Journal of HTAi HEALTH TECHNOLOGY ASSESSMENT INTERNATIONAL RWE4Decisions REAL WORLD EVIDENCE

International Journal of Technology Assessment in Health Care

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Policy

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Real-world evidence to support Payer/HTA decisions about highly innovative technologies in the EU—actions for stakeholders

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Table 2. Stakeholder roles in relation to use of RWD in decision making

HTA bodies/Payers	Identifying the questions that can/should be answered with RWD/RWE, providing guidance on critical assessment of RWE, collaborating with other stakeholders to collect, analyze, and interpret RWD, implementing innovative payment models requiring RWE generation
Regulators	Ensuring RWD collection strategies (such as registries) are multi-stakeholder and fit for regulatory purposes and where possible take account of the needs of HTA and Payers
Pharmaceutical industry	Actively engaging with other stakeholders to develop and implement plans for RWD collection, analysis, and reporting over the lifecycle of a highly innovative technology to generate robust RWE that helps resolve Payer/HTA decision uncertainties
Registry holders[a]	Involving all stakeholders in the establishment of registries and ensuring good governance that addresses data quality, accessibility, and sustainability to ensure the long-term value of registries and avoidance of waste in research
Clinicians and patients	Contributing real-world experience to support collection and generation of RWD that is useful and informs decision making at individual level, and population-level to create RWE
Patient groups	As authorized representatives of patients, engaging in co-creation of RWD/RWE, communicating the possible uses of RWD and good governance processes to encourage patient involvement
RWD/analytics groups[b]	Curation and analysis of RWD from electronic health records and other sources such as claims databases, government records etc. with a commitment to transparency, replicability, and principled database epidemiology

[a]EMA uses the term "registry coordinator"—a person or entity having a role in the overall coordination of a registry or of a platform of several registries. Registry holders can be patient groups, healthcare professionals, clinical institutes, and manufacturers.
[b]These groups could be within the health sector, academia, or private sector consultancies.

RWE – RIZIV-INAMI workshop Sep 10th 2020

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TRUST4RD
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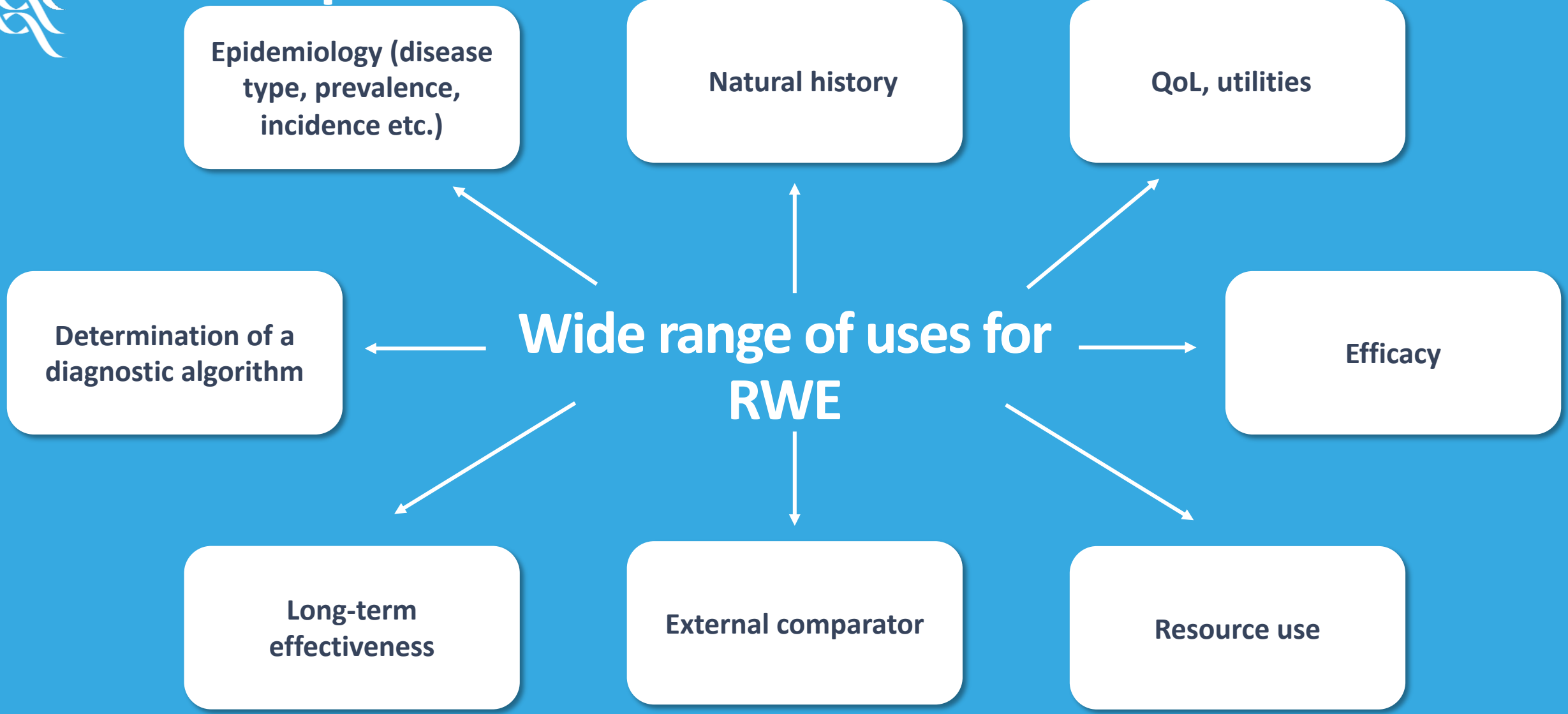
Workshop outcomes (1)



Workshop outcomes (2)

RWE4Decisions Case Studies Workshop - 10 September 2020

General Learnings Across All Case Studies to be used Publicly

- Highly innovative technologies are often developed for ultra-rare diseases and it is recognized that regulators are increasingly authorizing products on the basis of uncontrolled trials.
- HTA/Payers still prefer RCTs to demonstrate efficacy, but recognise that when uncontrolled trials are used, RWE will be an important part of the evidence submission, for example to create an external control arm to determine added clinical benefit
- RWE may have a role in resolving uncertainties that can't be resolved by clinical trials, but it is not meant to be a cheap substitution for a clinical study
- The objectives of TRUST4RD and RWE4Decisions is to identify evidence gaps that will remain after clinical trials and are likely to be important uncertainties for HTA/Payers and may be resolved by RWE

Workshop outcomes (3)

- Payers need to be clear about what data they require post HTA/reimbursement and collaborate to define a layered core dataset that identifies the essential (for all countries/decision makers), important and nice to have data (for local diseases)
- Registries should be disease based and coordinated across countries collecting data that is relevant for and can be accessed by population-level decision-makers such as HTA/regulators.
- As capture of in-hospital data improves, access to these data needs to be explored across national borders.

- Alignment with and avoidance of redundancies with other ongoing initiatives, e.g. parallel scientific advice, early dialogues, etc.

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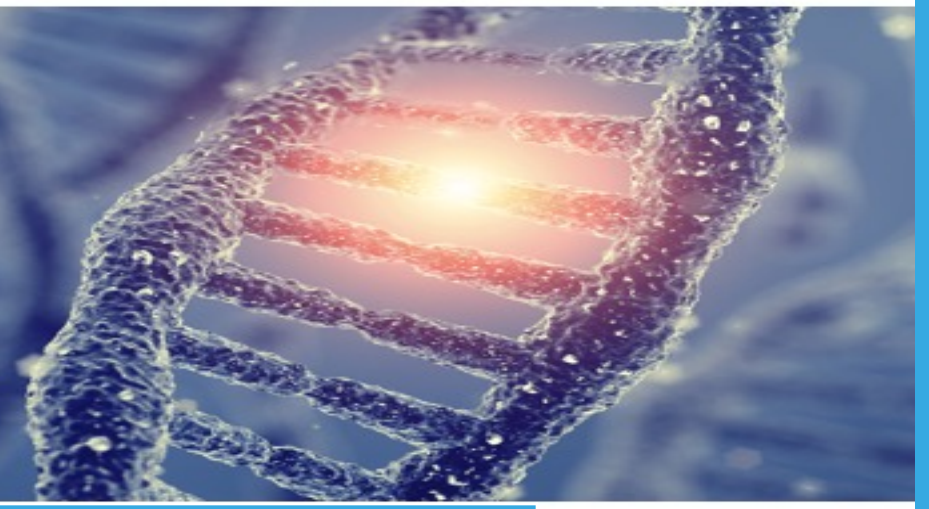
Call to Action



RWE4Decisions calls for a Multi-Stakeholder EU Learning Network on Real-World Evidence within the European Health Data Space

RWE4Decisions calls for the creation of a **multi-stakeholder EU Learning Network on Real-World Evidence**, which is based on a transparent governance mechanism. This Learning Network, designed for Member States to implement evidence-based decision-making, should be supported by EU funding, and:

1. clarify **when, by whom and how** real-world data should be collected in order to generate real-world evidence that **meets the needs of patients and healthcare systems;**
2. be based on a **voluntary mechanism**;
3. be underpinned by **robust methodologies** in alignment with other initiatives.

RWE4Decisions REAL WORLD EVIDENCE

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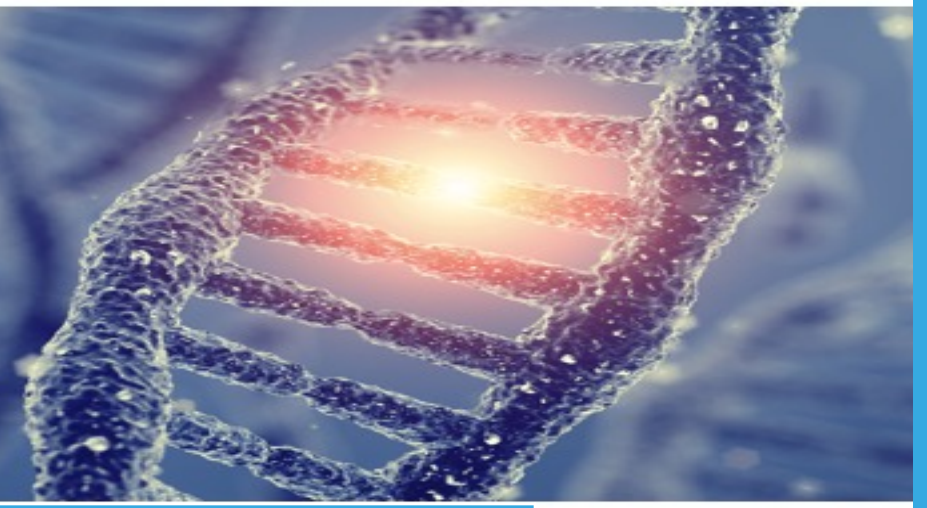

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Webinar
April 2021

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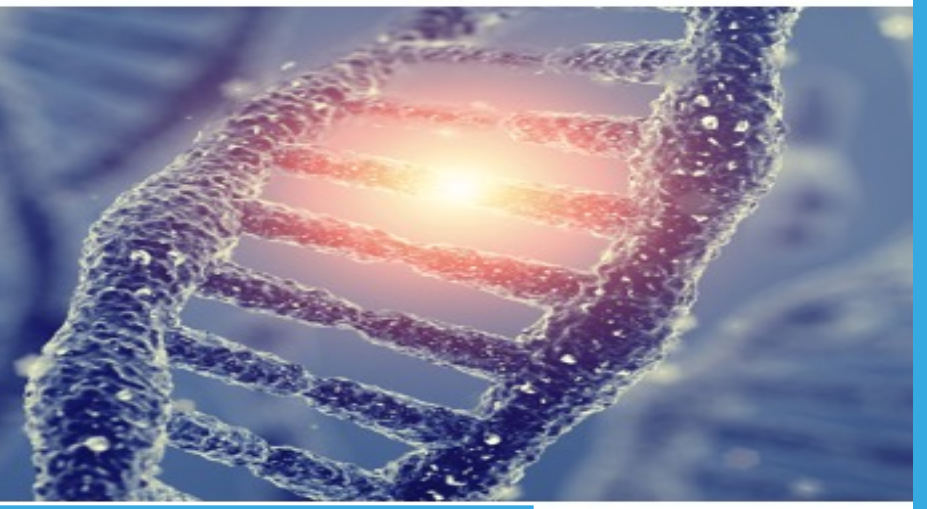

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Workshops
June 9th 2021
&
June 16th 2021

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Call to Action

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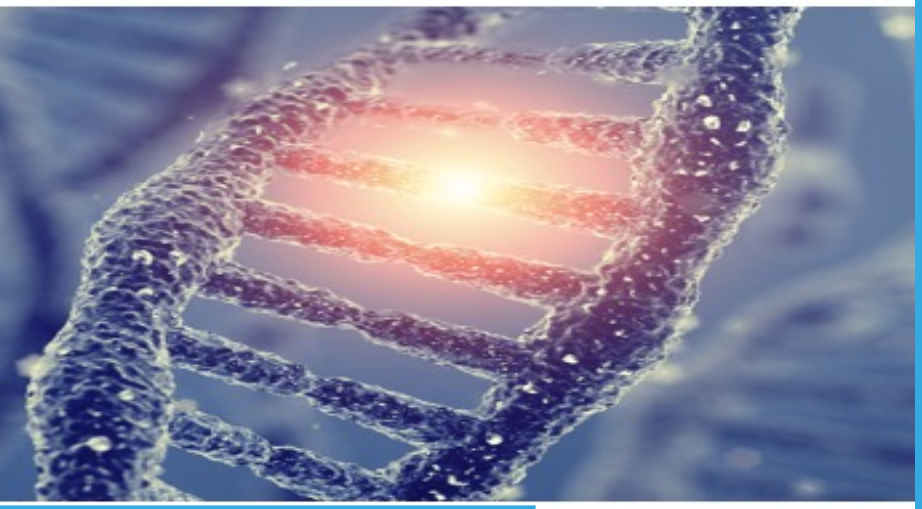

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Workshops

- **Evidence framework**
- **Checklist of RWE needs for reimbursement decision**
- **When Ob-MEA**
- **…**

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Thank you for your attention

Questions ?

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