RWE4Decisions REAL WORLD EVIDENCE

EVENT REPORT

# Co-Creating Real-World Evidence Excellence for Decision-Making: Meeting Regulatory and HTA/Payer Needs

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## HTA AND PAYER PANEL

Moderated by Dr Karen Facey, Consultant to INAMI-RIZIV

**Dr. Roisin Adams**
Ireland (NCPE)

**Dr. Antje Behring**
Germany (G-BA)

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Portugal (INFARMED)

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Italy (AIFA)

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Finland (FIMEA)

**The webinar addressed the value of collaboration between HTAs/Payers and Regulators, in particular in light of the European Medicines Agency (EMA) emerging guideline on registry-based studies and the proposal for DARWIN EU (Data Analytics and Real World Interrogation Network) within the EU Health Data Space. Consideration was also given to the use of real-world data (RWD)/ real-world evidence (RWE) in other regions.**

## Key Learnings on methodological guidance, registries and the use of RWD

### Benefits

- The EMA guideline on registry-based studies will clarify how the construct and analysis of registries can be improved. This will be helpful to HTA/Payers to maximise the value of RWD for benefit assessments, cost effectiveness assessments and pricing and reimbursement decisions.
- There is an increasing need to link high quality data collection, including for patient-relevant and clinical outcomes, to reimbursement systems.
- There is a need for linking financial incentives to data collection in registries and other forms of real-world data collection, to ensure quality, coverage and generalizability of results, especially when data are used for the evaluation of effectiveness.
- There is a need for awareness that standard epidemiological and statistical methods exist and if used appropriately, are acceptable for decision-making.

## Success stories of using RWE

An Italian registry helped develop a historical control arm for an orphan medicinal product. The single arm trial, supported by the registry data, enabled decision-making by the Italian Medicines Agency (AIFA) and the German Federal Joint Committee (G-BA).

Linking reimbursement decision to routine data collection in Italy and Portugal was proven successful in ensuring representativeness of RWD.

RWE alone supported positive marketing authorization by the EMA in one case.

RWD have also enabled granting of a conditional marketing authorization in some cases when RCT data alone were not convincing.

Use of RWE as a comparator to single arm trials led to successful market authorisation approvals.

## Outstanding Challenges

- Alongside the guidance on EMA registries, consideration of the duration and sustainability of registries is needed, as well as additional guidance on use of registry-based data for cost effectiveness and budget impact analysis and OBMEA.
- Guidance for regulators and HTA/Payers is also needed on the use of electronic health data arising from a range of sources, including methods for data curation, management and analysis, including specific research questions such as creation of external control group, long-term effectiveness etc.
- Disease registries may be preferred to multiple treatment/product/indication registries, but trade-offs arise given the multiple purposes of a disease registry.
- Clarity is needed on the questions that will be asked by HTA/Payers that can be answered or supported by RWD. Developers also have to make clear what kind of questions they would like to answer when they propose to use RWE to fill evidence gaps.
- Data collection in registries, routine healthcare or reimbursement systems comes with a major resource and governance burden, especially for the cross-country data collection.
- Guidelines are needed on how to standardize creation and use of RWD for HTA/Payer decision-making.
- Regional collaborations, such as FINOSE, undertake joint assessments but do not collaborate on data collection post-assessment.

## Conclusions

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| 1 | With the advent of high value, highly innovative technologies, HTA and Payer authorities may need to consider alternative reimbursement models. RWD can play an important role in reimbursement decision-making on a population and individual basis. |
| 2 | The aim is not to position RWE as a short-cut to substitute randomised controlled trials (RCTs) but a powerful complementary approach in addressing and resolving uncertainties and questions that cannot be answered with clinical trials. When possible, RCTs should be conducted as, by design, they are a preferred methodological approach to establishing efficacy and safety of new medicinal products. |
| 3 | Alignment on disease-specific data needs, quality, privacy and governance and operationalization of analytics remain an important issue requiring solutions, such as for example the establishment of an EU framework. |

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| 4 | Payers need to agree how they want to be involved in DARWIN EU, particularly in relation to asking research questions, training and methodological developments, and in relation to the policy and practice developments within the European Heath Data Space. Other stakeholders should also be integrated into the design of these collaborations. |
| 5 | Collaboration is needed to agree on approaches to critical assessment of RWD in clinical and cost effectiveness evaluations and if required, to align data collection requirements before and after a conditional reimbursement decision. There is a need to share use cases and learn from them, to determine what, when and how evidence needs to be generated to resolve decision-relevant uncertainties for HTA/Payers. A guidance that covers evidence generation based on various types of data collections systems beyond registries is needed for HTA/Payer bodies. |
| 6 | The proposed EU HTA Regulation on joint clinical assessment could play a role in promoting scientific consultations about use of RWD and voluntary collaboration in relation to data collection post HTA. |
| 7 | A multi-stakeholder approach is needed to address all these issues and the role of industry needs to be clarified. |

## Upcoming activities of the 'Learning Network'

Two subsequent webinars are planned for the Autumn, where other multi-stakeholder initiatives, methodological guidance and white papers will be explored to allow for a broader understanding of existing - and potentially still missing - elements for HTA/Payers to use RWD/RWE in decision-making.

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 h https://rwe4decisions.com/event/co-creating-rwe-excellence-for-decision-making-meeting-regulatory-and-hta-payer-needs/

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 June.