



4. Overcoming Obstacles: Charting the Path Ahead

- **Introduction, presentation of RWE4Decisions and other initiatives** François Meyer, Consultant, Special Advisor for RWE4Decisions
- **Stakeholder Actions to Generate Better Real-World Evidence for HTA/Payer Decisions** Piia Rannanheimo, Chief Specialist at Fimea (Finnish Medicines Agency)
- **Alignment of Evidence Needs with Study Designs and Data Sources** Massoud Toussi, VP, Global Head of RWE at Cytel
- **Discussants**
 - Jana Hlaváčová Patient advocate, European Multiple Sclerosis Platform
 - Niklas Hedberg, Chief Pharmacist, TLV, Sweden, Vice-chair of European HTA Coordination Group
 - Conor Teljeur, Chief scientist at HIQA Health Information & Quality Authority



RWE4Decisions, a Payer/HTA-led multi-stakeholder Learning Network

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Overcoming Obstacles: Charting the Path Ahead

ISPOR EU Real-World Evidence Summit 2024

17 November 2024

RWE4Decisions REAL WORLD EVIDENCE



RWE4Decisions: A payer-led initiative



- **RIZIV – INAMI : (National Institute for Health and Disability Insurance)**
‘Payer’ organisation : Federal public body of social security in Belgium.
- RIZIV – INAMI’s Pharmaceutical Policy Department
 - organises and directs the operation of the consultation bodies and their working groups
e.g. **the Commission for the Reimbursement of Medicines**
 - supports the operation of the sector and solves its problems
 - carries out scientific and statistical research and interprets information from databases.



INAMI multi-stakeholder roundtables – Exploring use of RWE for payer decision making about highly innovative medicines

The use of real world data throughout an innovative medicine's lifecycle

1. Introduction and objectives

The challenge for health policies is to provide high quality of care for all, within a sustainable health system. Innovations in healthcare such as innovative medicines play a crucial role in improving population's health. The way these medicines are developed, their price and their usage in daily practice can strongly impact on the quality and the sustainability of our health systems. Improved policies are needed to ensure timely and especially in areas of unmet need. Initiatives such as Adaptive Pathways pilot¹ and EarlyList Medicines (PRIM) However, the generation of evidence for these innovative rare diseases and for personalised medicine where the patient is the focus of care is a challenge.

There is an increased interest in the use of real world data evidence generation for innovative medicines.² It is expected that the generation of additional evidence post launch relation to the value of medicines and may optimise approach several challenges emerge, such as how to manage expectations how to better understand their usefulness and their price lifecycle (and not just post-launch), and how to encourage Review of current policies/perspectives from the Innovative initiative, it becomes clear that there is a need for common on the relevance of RWD, and harmonising the regulatory governance.

The purposes of this paper are

- to discuss the usefulness of RWD throughout the lifecycle providing realistic expectations about their possibilities
- to list the current issues in the collection, interpretation
- to propose principles of good practice and necessary throughout the lifecycle of innovative medicines.

Outcomes based pricing and reimbursement of innovative medicines with budgetary limitations

Discussion document for the multistakeholders meeting on pharmaceuticals (Meeting DG GROW 12th September 2017)

1. Introduction

Health policies in the EU aim to increase the healthy life expectancy of citizens within the limits of the available public resources. In order to achieve this objective, there is a need to improve the quality, effectiveness, and efficiency of EU health systems.¹

In addition, there is a continuous need for innovative health technologies, such as medicines, that help to substantially reduce morbidity and mortality, and improve quality of life.² However, these truly innovative technologies³ usually come at an extra cost, and – given the requirement for efficiency and sustainability – it is of key importance to establish appropriate methods and procedures for pricing and reimbursement (P&R) of these technologies.

The increasing focus in our healthcare systems on outcomes that matter for patients may create new opportunities in this regard. P&R decisions for innovative technologies that account for the added value that those technologies deliver for patients and society overall, will encourage the continued search for truly innovative technologies. Value can thereby be defined as "the importance, worth, or usefulness of something".⁴ It is recognised that the value of a new medicine is determined by both disease and treatment related characteristics.⁵ Indeed, if the impact of a disease on patients is high (severe symptoms, disability, reduced life expectancy etc.) and the medicine provides a substantial impact in reducing morbidity, improving quality of life or life expectancy, it can be considered of high value.



Tool for Reducing Uncertainties in the evidence generation for Specialised Treatments for Rare Diseases.

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TRUST4RD
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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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Objectives: There are divergent views on the potential of real-world data (RWD) to inform decisions made by regulators, health technology assessment (HTA) bodies, payers, clinicians, and patients. This RWE4Decisions initiative explored the particularly challenging setting of highly innovative technologies, which require Payer/HTA to make decisions on a small evidence base with major uncertainties. The aim was to go beyond strategic intent to consider actions that stakeholders could take to improve use of RWD in this setting. **Results:** Case studies of recent Payer/HTA decisions about highly innovative technologies were considered in light of recent international initiatives about RWD. This showed a lack of clarity about the Payer/HTA questions that could be assessed by RWD and how the quality of real-world evidence (RWE) could be assessed. All stakeholders would together to create a vision whereby stakeholders agree what RWD can be collected for highly innovative technologies based on principles of collaboration and transparency. For each stakeholder group, recommended actions to support the generation, analysis, and interpretation of RWD to inform decision making were developed. For HTA bodies, this includes cross border HTA/regulatory collaboration to agree RWD requirements over the technology life cycle to inform initial recommendations and assessment, data analytics methods development for HTA, and promotion of transparency in RWE studies. **Recommendations:** Stakeholders need to collaborate on demonstration projects to consider how RWE can be developed to inform healthcare decisions and contribute to a learning network that can develop systems to support a learning health system and improve patient outcomes through best use of RWD.

Background

Highly innovative technologies (such as immuno-, cell, and gene therapies that use novel molecular biology to target the underlying cause of a disease) aim to deliver transformative patient benefits. Where large potential benefits is expected in areas of high unmet medical need (such as rare diseases), regulators have developed expedited pathways. Regulatory approval based on interim analyses of short-term end points and data cut-offs is common, as is the use of uncontrolled trials. This means that some health technologies enter the market with a limited evidence base to demonstrate clinical effectiveness. There is substantial pressure for Payers (or health technology assessment (HTA) bodies) to make (or inform) pricing and reimbursement decisions about these technologies at the point of market launch. However, uncertainties exist about the population to be treated, natural history of disease, size, and durability of clinical effects compared to treatment alternatives, safety, cost effectiveness, and budget impact. As these highly innovative technologies often have a high price due to the complexity of development and projected high patient benefit, decisions about their value are challenging.

Although less than twenty cell and gene therapies had received regulatory approval by the end of 2018 in the EU, in early 2020 there were 1,000 clinical trials underway in over 400 countries (1). Therefore Payer/HTA need to quickly find ways to make their decision-making processes to help resolve uncertainties and mitigate risks. One option is to consider a longitudinal approach to evidence generation with collection of real-world data (RWD) over the life cycle of the technology (2).

The potential to use RWD to make decisions about highly innovative technologies links into the major scientific and technological advancements in digital health (4,5). Numerous national and transnational collaborations are expected to contribute to improvements in quality, coverage, and access to RWD (6–8) and have the potential to create learning health systems that will improve patient outcomes (9). The health technology industry, health systems,

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TRUST4RD
Tool for Reducing Uncertainties in the evidence generation for Specialised Treatments for Rare Diseases

RWE4Decisions recommended actions for stakeholders to support payer/HTA decisions about highly innovative technologies

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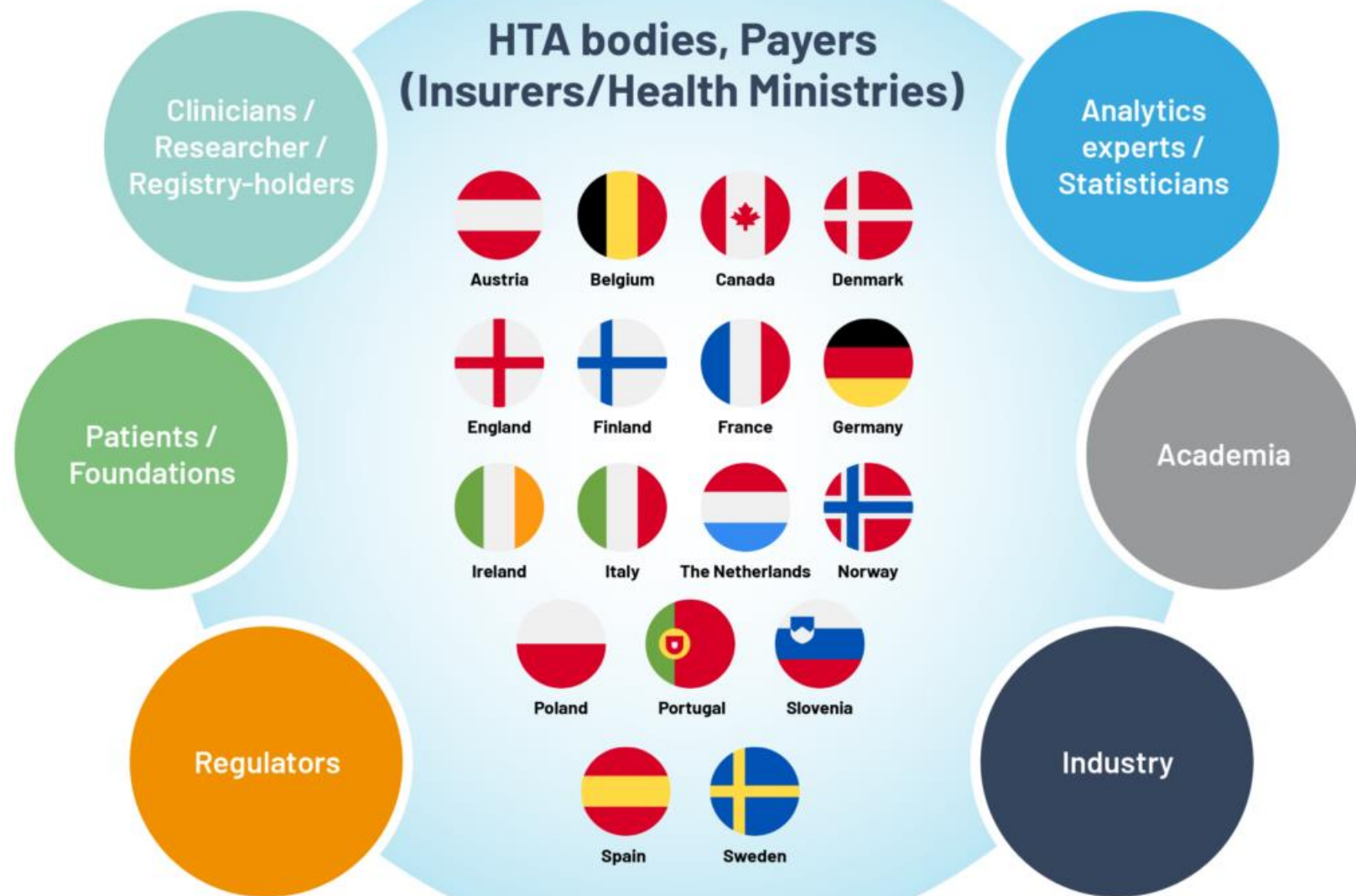


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The Learning Network



Secretariat **FIPRA**

Engaging in dialogue



Context: the HTA regulation

Joint Scientific Consultation (JSC)	Joint Clinical Assessment (JCA)
DEFINITION	
<ul style="list-style-type: none">- Scientific advice- provided jointly by HTA bodies- Can be in parallel with regulators- To HTD on the clinical development	<ul style="list-style-type: none">- Joint HTA reports, produced by 2 EU MS- On HTD submission dossier- HTD cannot submit data again on national level- Focussing on the clinical domains- Without value judgements- MS to give due consideration
AIM	
To generate evidence that satisfies the needs of HTA bodies during their assessment and ultimately facilitates patient access	To avoid duplications of work at the national level, increase consistency and quality of assessments and ultimately facilitate patient access

HTD = Health Technology Developer



RWE and the HTA regulation

- **Joint Clinical Assessments**

- **RWE submission** at the time of initial assessment?
- **Methodological guidance** by HTAR Coordination Group :
Chapter on RWE

- **Joint Scientific Consultations**

- Dialogue between pharma company and HTA bodies (possibly in parallel with EMA) at an **early stage** of product development
- Includes discussion on possible evidence gaps and planned **Post Launch Evidence Generation (PLEGs)**



European and Regional initiatives



- Joint HTA of medicinal products in the 5 Nordic countries: Denmark, Finland, Norway, Sweden, Iceland
- Successor of FINOSE



- Multi-country collaboration in horizon scanning, information sharing, HTA, pricing and reimbursement
- Initiative for improved and sustainable access to innovative treatments for patients of participating countries: Belgium, Netherlands, Luxembourg, Ireland, Austria



- Data Analysis and Real World Interrogation Network
- Coordination centre created by the European Medicines Agency and the European Medicines Regulatory Network, provide evidence on medicines drawn from real-world healthcare databases across the EU



Other European initiatives



- IDERHA - Integration of Heterogeneous Data and Evidence towards Regulatory and HTA Acceptance
- European public-private partnership launched in April 2023
- Aims to be an open, disease agnostic, federated pan-European data space for collection, access, use and reuse of digital health data



- Horizon Europe Consortium aiming to promote use of RWD in regulatory decision-making and HTA
- Making use of technologies like AI, machine learning



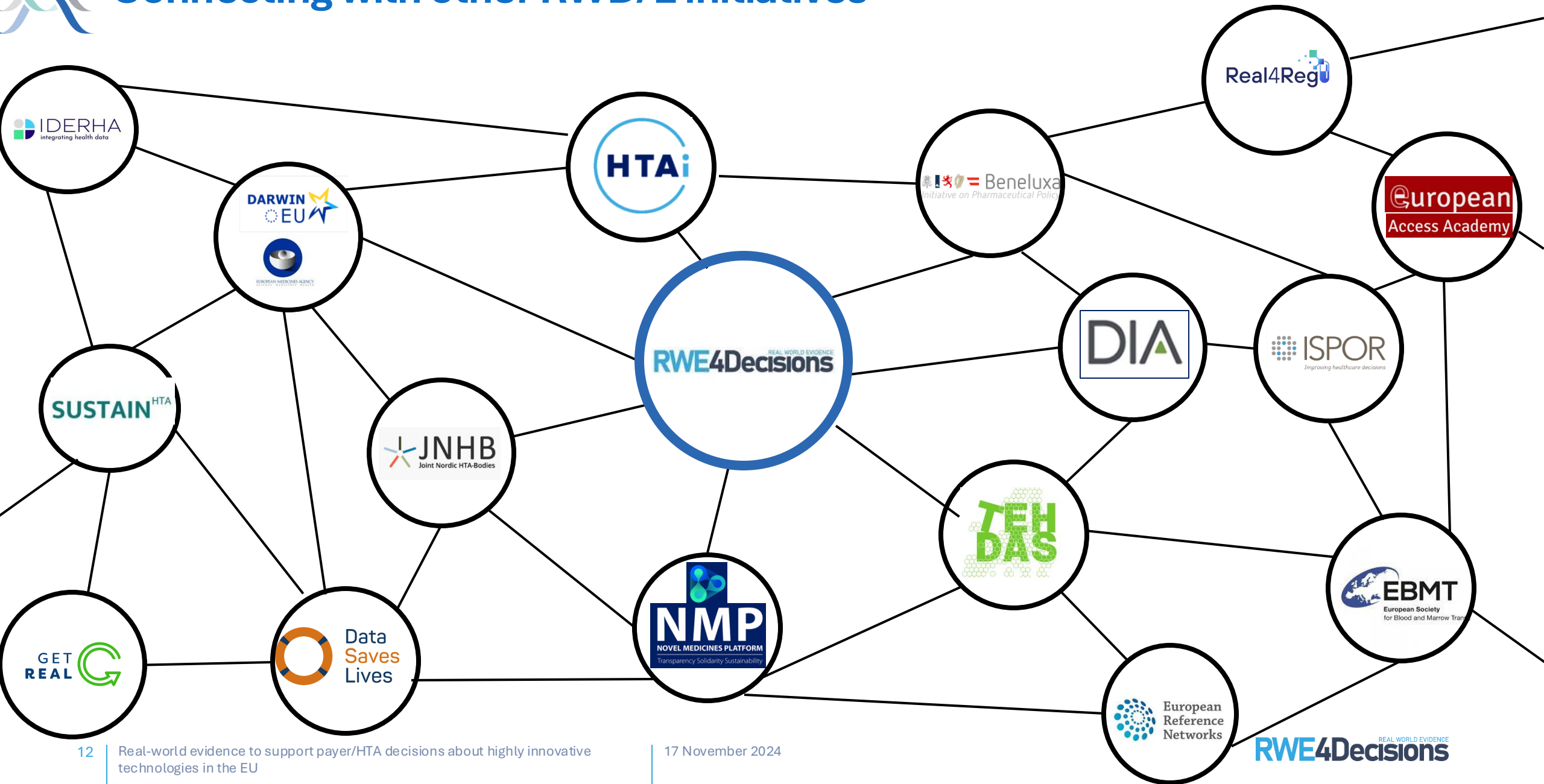
- Multi-stakeholder initiative led by the European Patients' Forum (EPF) and the European Institute for Innovation Through Health Data (i~HD), aiming to raise awareness about the importance of health data and improve understanding of its use.



- Set by the Innovative Health Initiative (IHI)
- Multi-stakeholder forum advancing use of RWE in regulatory, HTA and clinical decision-making. Platform for collaboration and knowledge sharing.



Connecting with other RWD/E initiatives





Thank you!

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