CORD webinar – Matching Access to Risk and Getting Real

Early Access & RWE: building trust and reducing stakeholder uncertainties – a European perspective

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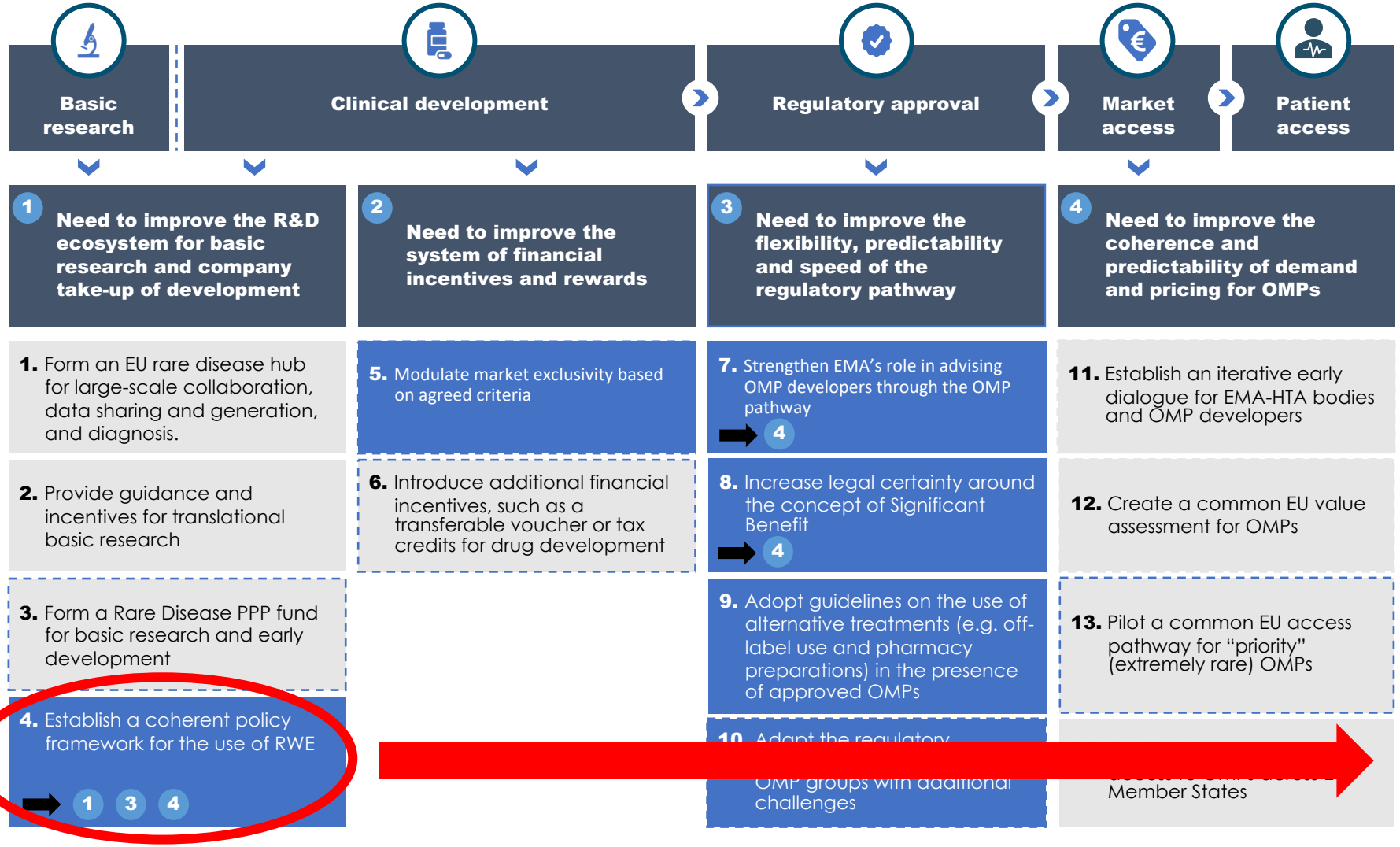
Toon Digneffe, Head EU Public Affairs & Public Policy, Takeda

toon.digneffe@takeda.com

European Expert Group on OD Incentives

14 policy proposals for Orphan Medicinal Product incentives



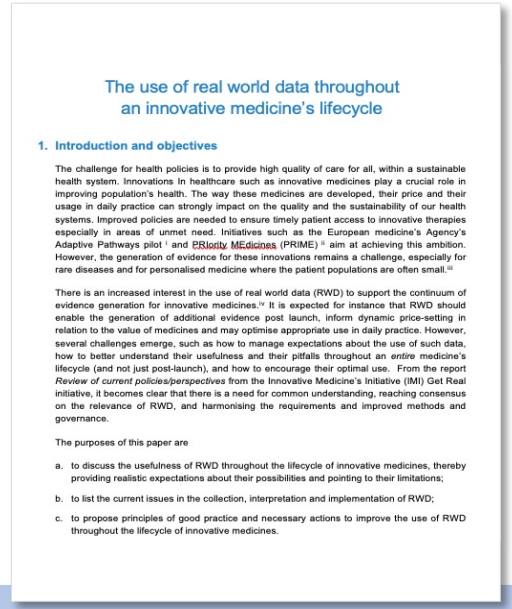
These proposals pursue or open up for a modulated approach to OMP incentives

These proposals can be addressed through the revision of the OMP Regulation

Copenhagen Economics CE

European Expert Group on OD Incentives

A history of multi-stakeholder collaboration on RWE



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 patient access to innovative therapies especially in areas of unmet need. Initiatives such as the European medicine's Agency's Adaptive Pathways pilot [i] and PRIority MEdicines (PRIME) [ii] aim at achieving this ambition. However, the generation of evidence for these innovations remains a challenge, especially for rare diseases and for personalised medicine where the patient populations are often small.[iii]

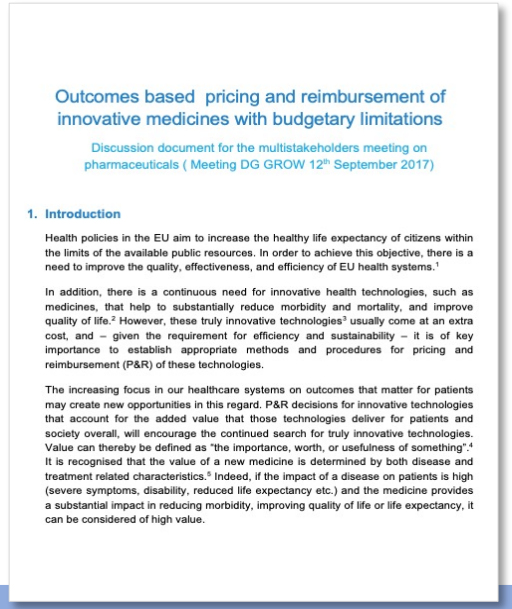
There is an increased interest in the use of real world data (RWD) to support the continuum of evidence generation for innovative medicines.[iv] It is expected for instance that RWD should enable the generation of additional evidence post launch, inform dynamic price-setting in relation to the value of medicines and may optimise appropriate use in daily practice. However, several challenges emerge, such as how to manage expectations about the use of such data, how to better understand their usefulness and their pitfalls throughout an *entire* medicine's lifecycle (and not just post-launch), and how to encourage their optimal use. From the report *Review of current policies/perspectives* from the Innovative Medicine's Initiative (IMI) Get Real initiative, it becomes clear that there is a need for common understanding, reaching consensus on the relevance of RWD, and harmonising the requirements and improved methods and governance.

The purposes of this paper are

a. to discuss the usefulness of RWD throughout the lifecycle of innovative medicines, thereby providing realistic expectations about their possibilities and pointing to their limitations;

b. to list the current issues in the collection, interpretation and implementation of RWD;

c. to propose principles of good practice and necessary actions to improve the use of RWD throughout the lifecycle of innovative medicines.



Outcomes based pricing and reimbursement of innovative medicines with budgetary limitations

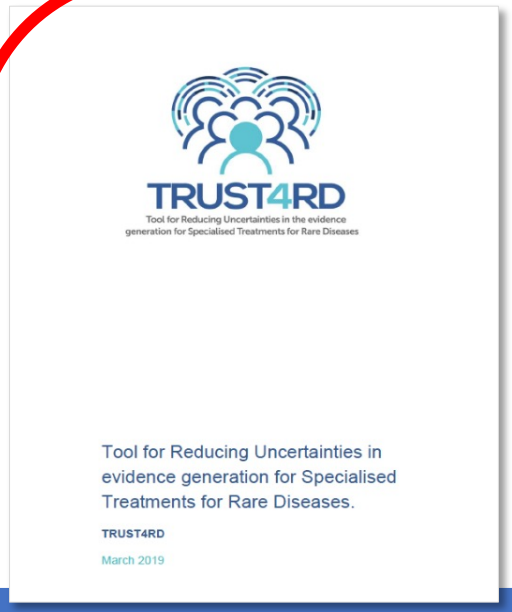
Discussion document for the multistakeholders meeting on pharmaceuticals (Meeting DG GROW 12th September 2017)

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 technologies[3] 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.



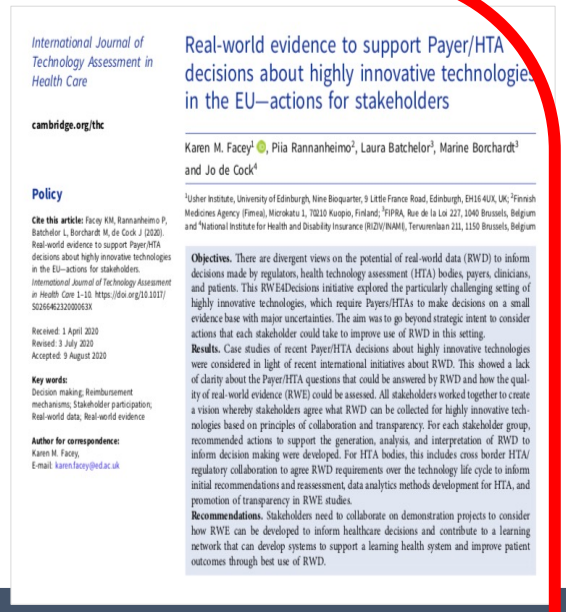
TRUST4RD

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

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

TRUST4RD

March 2019



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Policy

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Author for correspondence:
Karen M. Facey,
E-mail: karen.facey@ed.ac.uk

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

Karen M. Facey[1], Piia Rannanheimo[2], Laura Batchelor[3], Marine Borchardt[3] and Jo de Cock[4]

[1]Usher Institute, University of Edinburgh, Nine Bioquarter, 9 Little France Road, Edinburgh, EH16 4UX, UK; [2]Finnish Medicines Agency (Fimea), Microkatu 1, 70210 Kuopio, Finland; [3]IPRA, Rue de la Loi 227, 1040 Brussels, Belgium and [4]National Institute for Health and Disability Insurance (RIZIV/INAMI), Tervurenlaan 211, 1150 Brussels, Belgium

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 Payers/HTAs to make decisions on a small evidence base with major uncertainties. The aim was to go beyond strategic intent to consider actions that each stakeholder 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 answered by RWD and how the quality of real-world evidence (RWE) could be assessed. All stakeholders worked 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 reassessment, 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.

2016	2017	2018	2020
The use of real world data throughout an innovative medicine's lifecycle [Link]	Outcomes based pricing and reimbursement of innovative medicines with budgetary limitations [Link]	TRUST4RD – Tool for Reducing Uncertainties in the evidence generation for Specialised Treatments for Rare Diseases [Link]	RWE4Decisions recommended actions for stakeholders to support payer/HTA decisions about highly innovative technologies [Link]

Matching Access to Risk and Getting Real: from TRUST4RD to RWE4Decisions

From

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

2018/2019

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

- **Early and iterative dialogues** – win-win solutions for all stakeholders
- **Taxonomy of evidence gaps** – speaking a common language
- **Building trust** between stakeholders and in RWE as solution to reduce uncertainties

to

RWE4Decisions REAL WORLD EVIDENCE

2020/2021

RWE4Decisions – Real World Evidence to support HTA/payer decisions about highly innovative technologies

- Setting up a **Learning Network on RWE** involving policy makers, HTA bodies, payers, regulatory agencies, clinicians, patient groups, industry and academics experts

Multi-stakeholder participation

Wider stakeholder community

Public research bodies

Clinicians/relevant ERNs

Relevant EURORDIS members

EC/ EMA

INAMI-RIZIV

Thought leadership
(INAMI-RIZIV CEO Jo De Cock)

Multi-stakeholder Steering Group

HTAs
KCE, FIMEA, NICE

Patients
EURORDIS, EPF, ECPC

Academic
University of Edinburgh

Industry
EUCOPE & member companies

Clinicians/Researchers
EORTC, ERNs

Supported by RWE4Decisions Secretariat

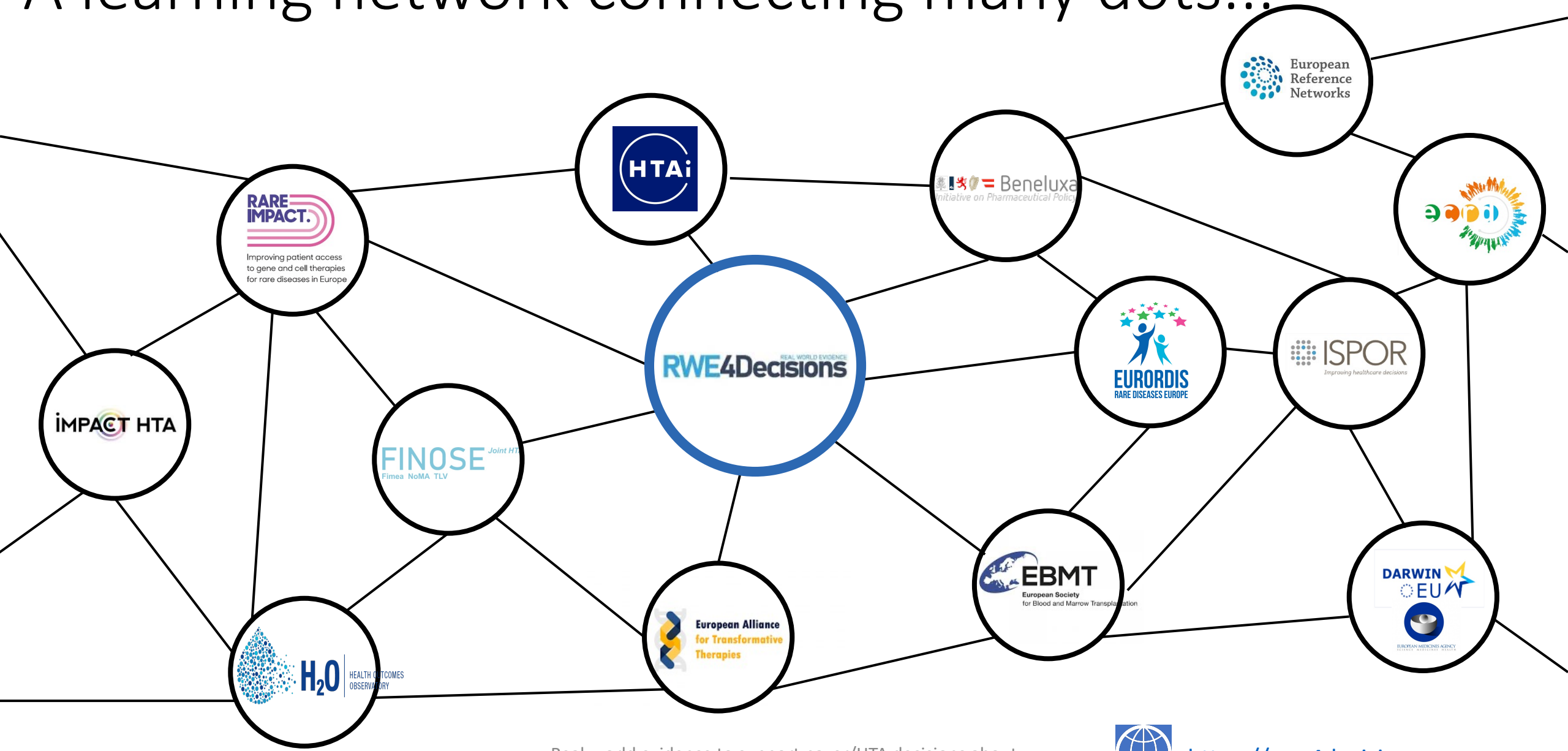
FIPRA

HTA/payer community

G-BA, AIFA, HAS, INFARMED, NICE, FIMEA (Connection to FINOSE), ZIN, KCE, NoMA, NCPA, TLV, AOK, Slovenian health insurers, Austrian insurers

CADTH

A learning network connecting many dots…



Real-world evidence to support payer/HTA decisions about highly innovative technologies in the EU

https://rwe4decisions.com/

2021 Agenda

RWE4Decisions 2021

Workstream 1: Case studies workshops (INAMI hosted)

Payer-led multi-stakeholder dialogues

- to develop a RWE generation framework to resolve Payer decision uncertainties at the time of launch for two types of highly innovative technologies
- to facilitate alignment in construction of Outcomes-Based Managed Entry Agreements

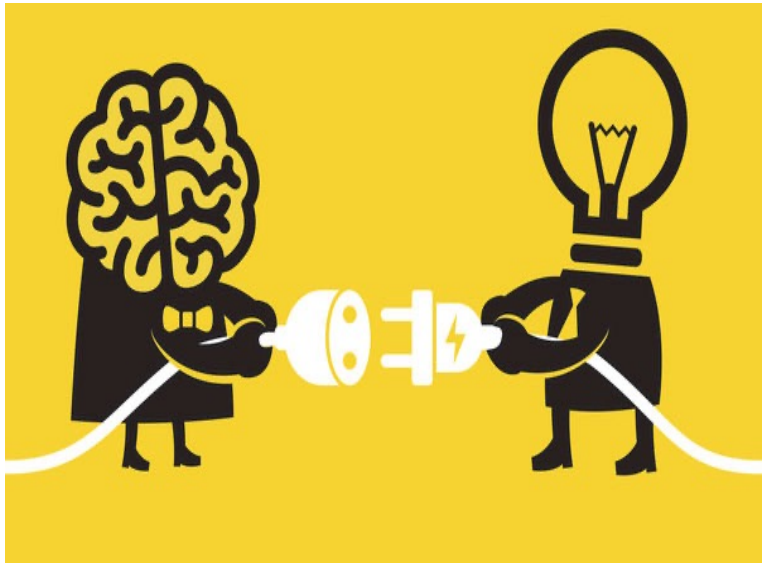
Workstream 2: Webinars

Putting the RWE4Decisions Learning Network into practice through a series of webinars for HTA/payers, EMA and stakeholders to exchange on methodological or practical questions about the planning, collection, interpretation and use of RWD in decision-making

Workstream 3: Advocacy

Advocating for a sustainable multi-stakeholder Learning Network on RWE within the European Health Data Space, and share learnings with EU and national policy-makers

What do we need to make progress ?



Barriers:

- Lack of trust between stakeholders
- Not speaking the same language
- Facing uncertainty linked to evidence gaps

Enablers:

- Multi-stakeholder collaboration building trust
- Common framework and language
- Tools to manage uncertainties

Missing elements:

- Operating EU Health Data Space
- Accessible and interoperable health data sets (role for ERNs)
- Functioning value-based healthcare framework (role for outcomes-based managed entry agreements)

Thank you!

toon.digneffe@takeda.com

RWE4Decisions: A payer-led initiative to develop a multi-stakeholder Learning Network about use of RWE for highly innovative technologies

WHAT?

Pragmatic and agile **Learning Network on RWE** including HTA bodies/payers, the EMA, patient representatives, researchers, clinicians, industry and academics...

To improve evidence-informed decisions for market access and reimbursement of highly innovative technologies

WHY DISTINCTIVE?

Payer-led

Multi-stakeholder in approach

- Actions for each stakeholder group
- Identification of gaps in knowledge of each stakeholder group
- Joint discussion of challenges and potential solutions
- Collaboration
- Transparency

WHY?

Encourage development of robust RWE to help optimize use of highly innovative technologies

To address the operational, technical and methodological gaps

ADDED VALUE?

Practical multi-stakeholder learnings on the potential use of RWE through a **'learning by doing' approach**

Share experience, pool resources, build trust