



Optimising Real-World Evidence to Deliver Value in Healthcare

23 September | 09:00 – 14:00 CEST |
Location: FEB, Brussels and online



Welcome remarks



Laura Batchelor
Managing Partner,
FIPRA International



Karen Facey
Senior HTA Adviser, RWE4Decisions;
University of Edinburgh

RWE4Decisions genesis: INAMI papers

Foundations for Consideration of 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 pilot¹ and PRIME² 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.^{3,4}

There is an increased interest in the use of real world data (RWD) to support the continuum of evidence generation for innovative medicines.^{5,6} 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

- to discuss the usefulness of RWD throughout the lifecycle of innovative medicines, thereby providing realistic expectations about their possibilities and pointing to their limitations;
- to list the current issues in the collection, interpretation and implementation of RWD;
- to propose principles of good practice and necessary actions to improve the use of RWD throughout the lifecycle of innovative medicines.

2016

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

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.

2017

Outcomes based pricing and reimbursement of innovative medicines with budgetary limitations



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

TRUST4RD

March 2019

2018

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

International Journal of Technology Assessment in Health Care

cambridge.org/thc

Policy

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

Karen M. Facey¹, Pia Rannanheimo², Laura Batchelor³, Marine Borchardt⁴ and Jo de Cock⁴

¹Usher Institute, University of Edinburgh, Nine Bioquarter, 9 Little France Road, Edinburgh, EH16 4UX, UK; ²Finnish Medicines Agency (Fimea), Mikaelinkatu 1, 00210 Kuopio, Finland; ³PIPPA, Rue de la Loi 227, 1040 Brussels, Belgium and ⁴National Institute for Health and Disability Insurance (RIZIV/INAMI), Tennoorenlaan 211, 1150 Brussels, Belgium

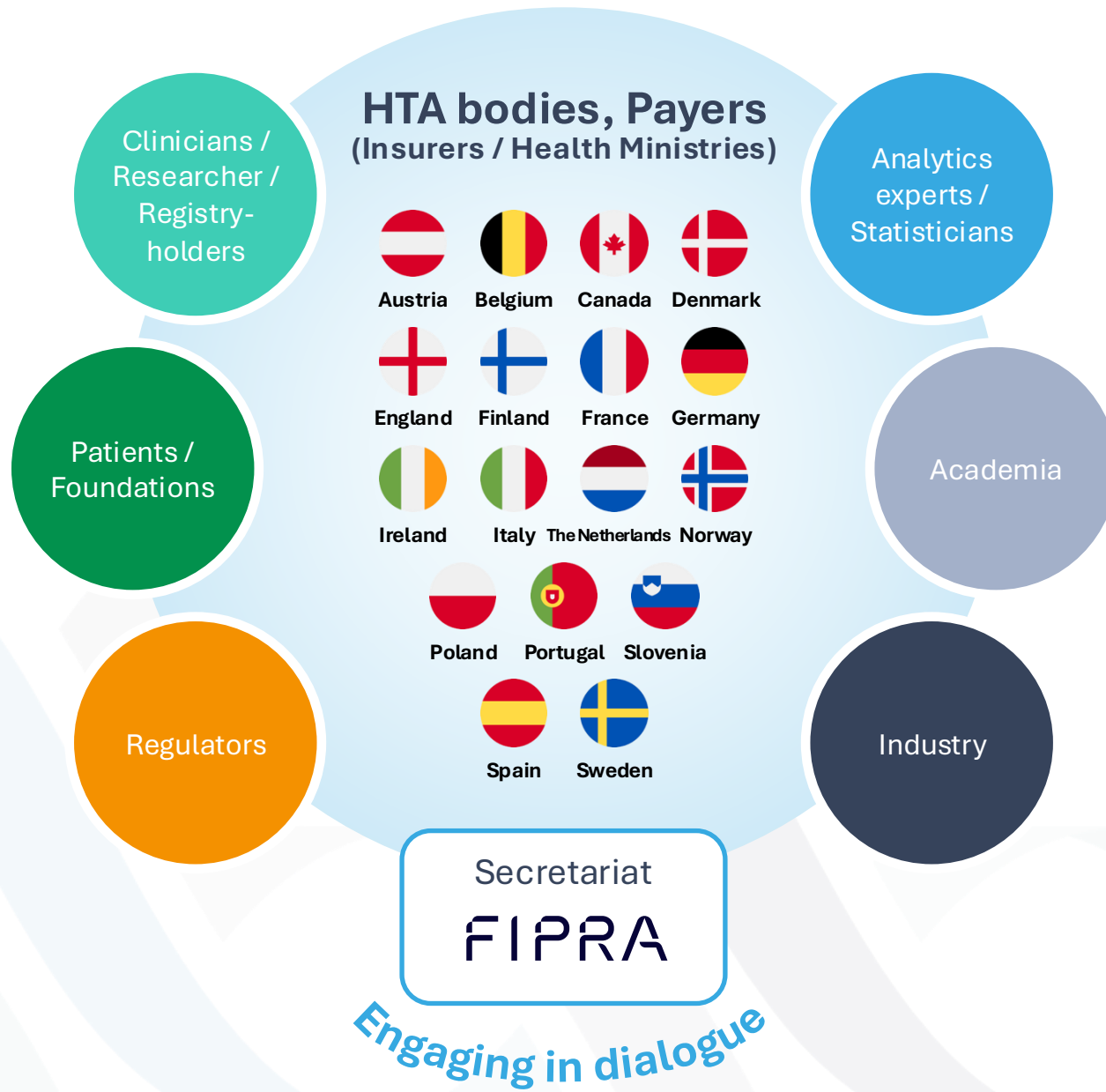
Objectives. There are divergent views on the potential of real-world data (RWD) to inform decisions made by regulators, health technology assessment (HTA) bodies, payers, clinicians, and patients. This RWE4Decisions initiative explored the particularly challenging setting of highly innovative technologies, 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.

2019/2020

RWE4Decisions first series of recommendations of actions for stakeholders to support payer/HTA decisions about highly innovative technologies



HTA/Payer-led, multi-stakeholder Learning Network

- | **Horizon scanning** systems that identify medicines which are most likely to need RWE
- | Identifying **what RWE is** needed to inform HTA/Payer decisions
- | Clarifying **how RWE should be generated** by stakeholders and evaluated by HTA/Payers¹
- | Aligning planning and execution of effective **Post Launch Evidence Generation (PLEG)** studies.

¹ Jaksa, A. A., Pavel, A. N., Aapro, M., Hedberg, N., Hodgkinson, V., Lambert, L. J., ... Facey, K. M. (2025). **Actions for Stakeholders to Develop Better Real-World Evidence for HTA/Payer Decision-Making**. *IJTAHC*, 1–32. doi:10.1017/S0266462325100238

REAL WORLD EVIDENCE

RWE4Decisions

2026 Steering Group

HTA BODIES / PAYERS / INSURERS



Niklas Hedberg
TLV



Piia Rannanheimo
Fimea



Cláudia Furtado
INFARMED



Christian Dehlendorff
Danish Medicines Council



Shaun Rowark
NICE



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Rotterdam University



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Calgary University, Registry developer

INDUSTRY



Moderators



Karen Facey

Senior HTA Adviser,
RWE4Decisions;
University of Edinburgh



Hans-Georg Eichler

Consultant Physician,
Association of
Austrian Social
Insurance Institutions



Eric Sutherland

Senior Health
Economist, OECD



Shaun Rowark

Associate Director
Healthcare Data
Analytics, NICE,
England

Optimising RWE to Deliver Value in Healthcare

Agenda

09:15 – 09:20 ●

Welcome remarks

09:20 – 10:25 ●

**Navigating Europe's Future –
Opportunities for RWE Innovation?**

10:25 – 11:10 ●

The European Health Data Space

11:10 – 11:40 ●

Break

11:40 – 12:20 ●

Artificial Intelligence (AI) for RWE Generation

12:20 – 12:55 ●

**RWE4Decisions Priorities to Support Better
RWE Generation for HTA/Payer Decision**

12:55 – 13:00 ●

Closing remarks

13:00 – 14:00 ●

Networking lunch

Principles of engagement



Meeting will be recorded for sharing on the RWE4Decisions website and in the report.



In the room, please raise your hand to ask a question or QR code.
Online, please use the **Slido for questions**, using this QR code.



Adherence to anti-corruption and anti-bribery rules

Participants must not make any direct or indirect promise or payment that could lead to a governmental act or decision helping parties obtain/retain business or enjoy any kind of preferential treatment.

Compliance with National and European Competition Laws

Participants must not engage in any discussion about or sharing of commercially sensitive information, e.g. net **prices** (including discounts and rebates), future list prices, customers, costs, strategic plans and output.

09:20 – 10:25

Navigating Europe's Future - Opportunities for RWE Innovation?



Hans-Georg Eichler

Moderator

Chief Consultant Physician, Association of Austrian Social Insurance Institutions

Slido

Ask your question



Roisin Adams

Head of HTA Strategy,
National Centre for
Pharmacoeconomics,
Ireland



Kristin Thompson

Investment Director
(Life Sciences), Merieux
Equity Partners



Alexander Natz

Secretary General,
EUCOPE



Niklas Hedberg

Chief Pharmacist, TLV,
Sweden



Wim van Harten

Research Group leader,
Netherlands Cancer
Institute

09:20 – 09:35

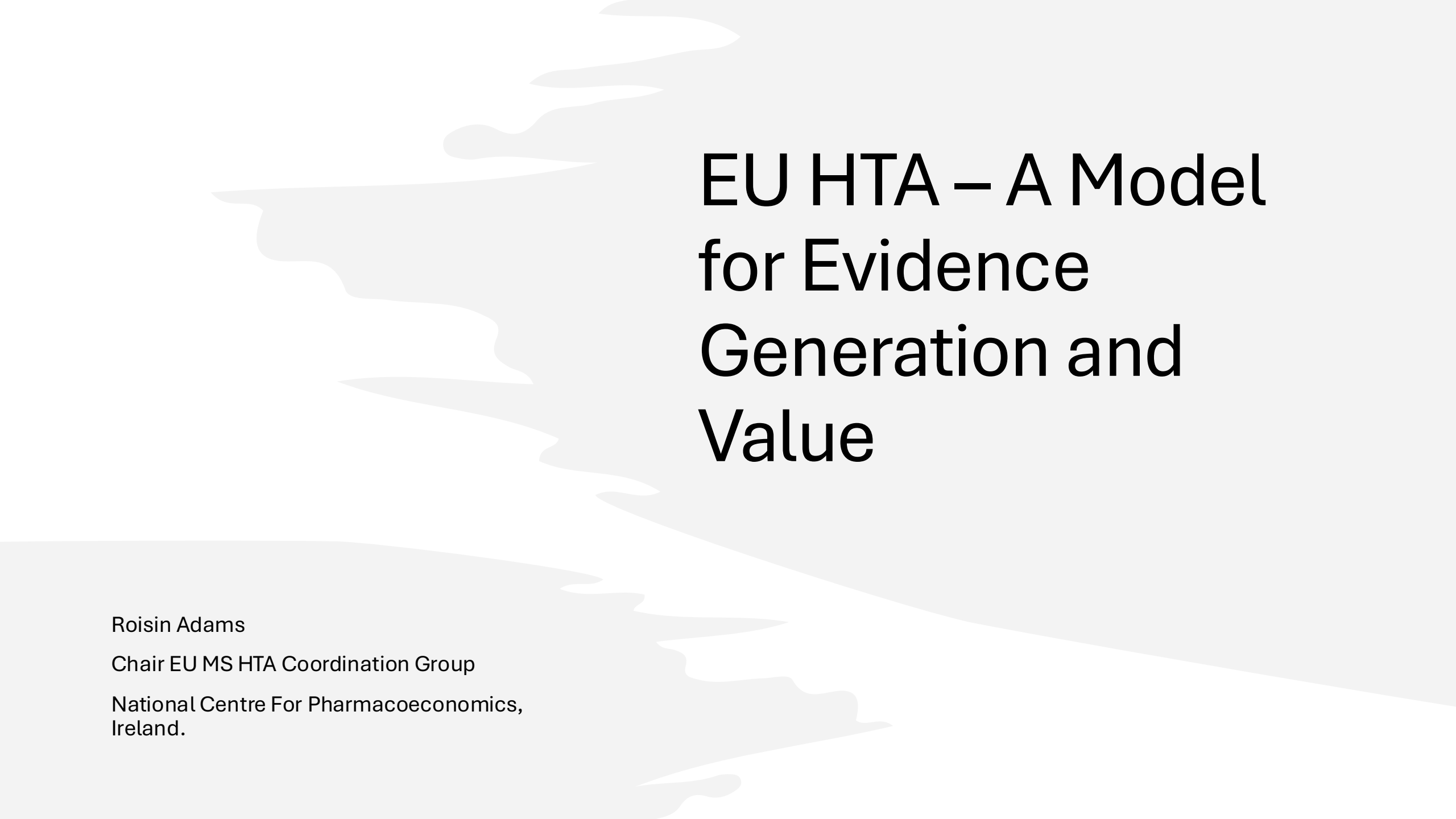


Keynote address: Implementing the EU HTA Regulation

Roisin Adams

**Chair of the EU Member State HTA
Coordination group**

**Head of HTA Strategy, National Centre
for Pharmacoeconomics, Ireland**

A light gray, stylized map of Europe is positioned in the background of the slide, centered behind the title text.

EU HTA – A Model for Evidence Generation and Value

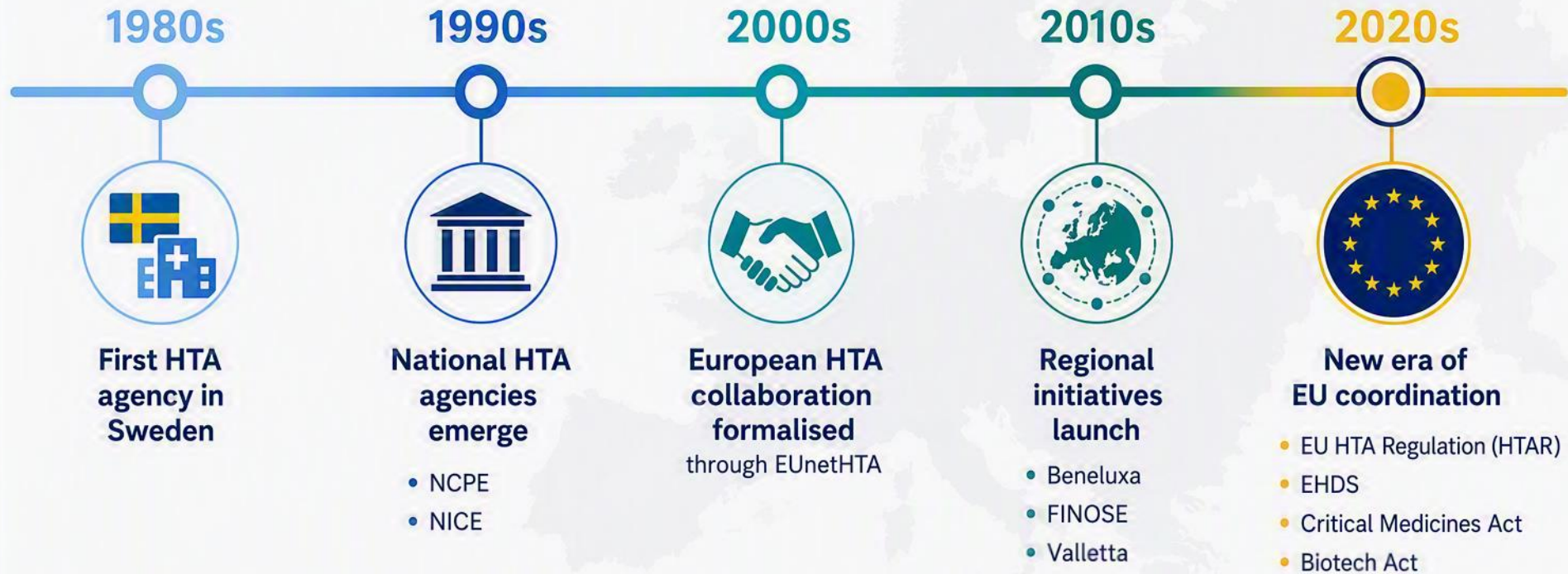
Roisin Adams

Chair EU MS HTA Coordination Group

National Centre For Pharmacoeconomics,
Ireland.

HTA & Value in Europe

The European HTA Story



Since 2025: Joint Clinical Assessments

15 Joint Clinical Assessments

- 11 Oncology
- 3 ATMP

4 Published

- 3 Oncology
- 1 ATMP

4 Discontinued

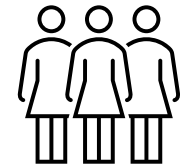
- 4 Oncology
 - 1 Withdrawn
 - 3 Insufficient data

Since 2025: Joint Scientific Consultations

4
HTACG &
EMA

3
HTACG

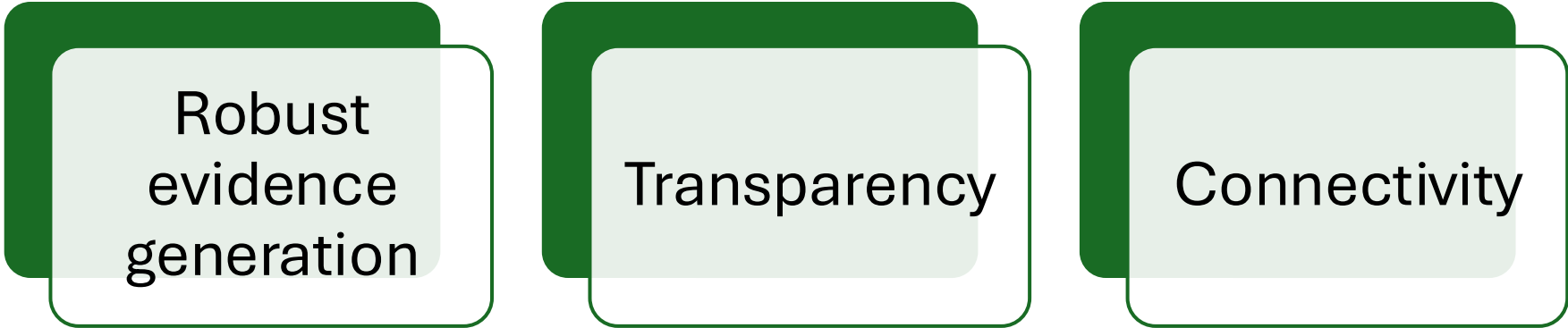
7 in 2025



10 patients
and clinical
experts

Workplan is to complete 8-12 JSCs for MPs in 2026

What does EU HTA offer?



Robust
evidence
generation

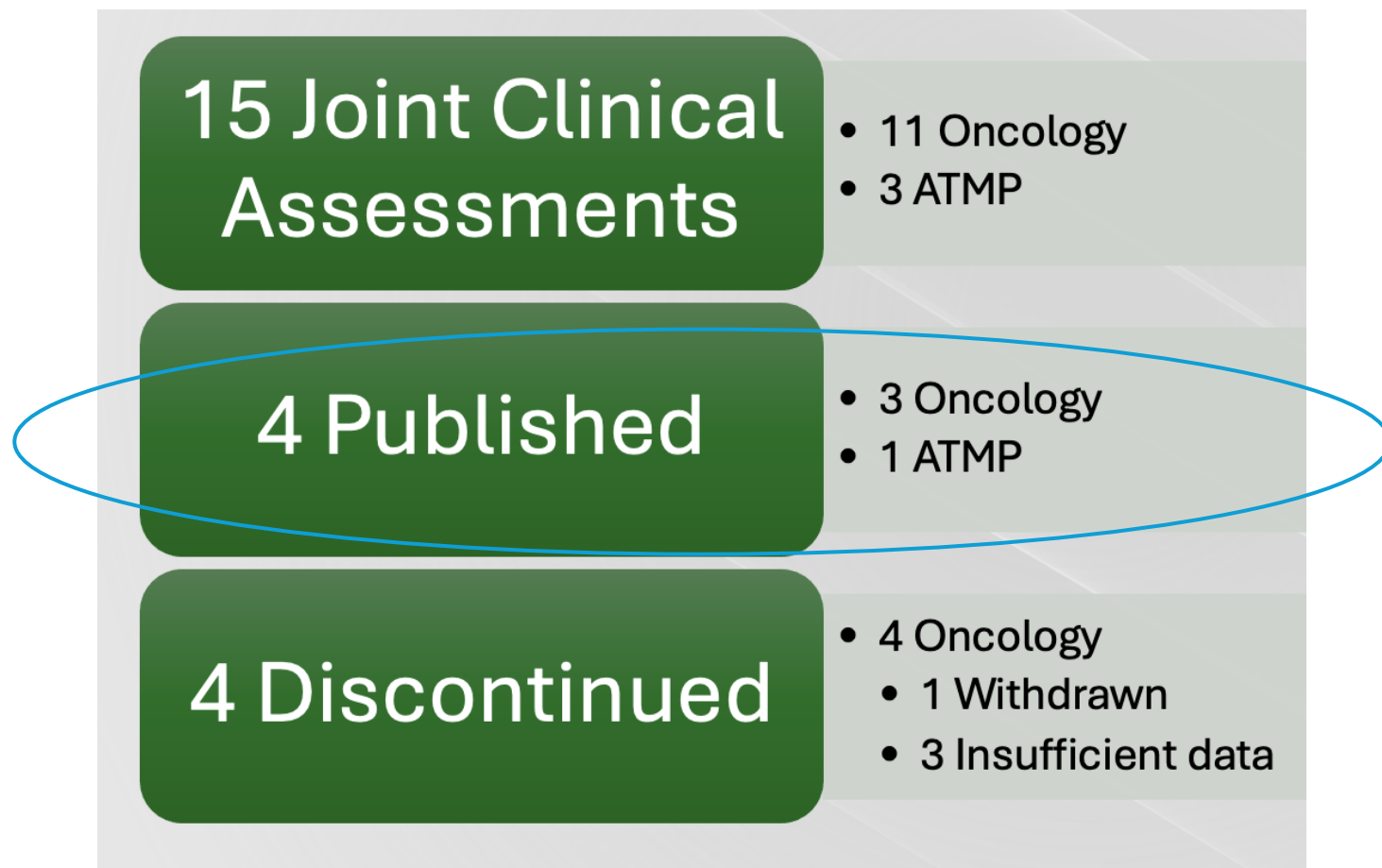
Transparency

Connectivity

Evidence Generation

- EU HTA provides the evidence framework (PICOs & Guidance)
 - Policy questions that payers need to be addressed
 - Creates a roadmap to the value proposition
 - Identifies populations of particular interest
- Joint Scientific Consultation can provide the earlier advice on how this is addressed

Transparency



Joint Clinical Assessment Report

Tovorafenib

Endorsed
2021/228.

Version 1.0

Endorsed by the Coordination Group pursuant to Article 12(2) of Regulation (EU) 2021/2282
on 30 April 2026

Dossier of the Joint Clinical Assessment
(JCA) of a Medicinal Product (MP)

Tovorafenib (Ojemda®)

Ipsen Pharma

Code: JCA-MP-2024-06

Version 1.0

Date submitted: 4 June 2026

Pur

**ANNEX TO THE JOINT CLINICAL
ASSESSMENT REPORT**

ON

TOVORAFENIB

**Factual accuracy check by the Health
Technology Developer
and response by the assessors**

Endorsed
2021/228.

Endorsed by the Coordination Group pursuant to Article 12(2) of Regulation (EU) 2021/2282
on 30 April 2026

Transparency – what else is published?

**15 Joint Clinical
Assessments**

- 11 Oncology
- 3 ATMP

4 Published

- 3 Oncology
- 1 ATMP

4 Discontinued

- 4 Oncology
- 1 Withdrawn
- 3 Insufficient data

Transparency

15 Joint Clinical Assessments

- 11 Oncology
- 3 ATMP

4 Published

- 3 Oncology
- 1 ATMP

4 Discontinued

- 4 Oncology
- 1 Withdrawn
- 3 Insufficient data

The table below lists the information, data, analyses and other evidence that were requested by the Commission in its second request referred to in Article 10(5) of Regulation (EU) 2021/2282 and that were not submitted by the health technology developer Advenchen Laboratories, LLC in the updated dossier (V0.2) of the joint clinical assessment of the medicinal product Catequentinib. Updated dossier submission date: 9 April 2026.

The dossier includes information on two therapeutic indications for the treatment of synovial sarcoma and for the treatment of leiomyosarcoma, referred to as indication a and indication b in the below table.

Transparency

The table below lists the information, data, analyses and other evidence that were requested by the Commission in its second request referred to in Article 10(5) of Regulation (EU) 2021/2282 and that were not submitted by the health technology developer Advenchen Laboratories, LLC in the updated dossier (V0.2) of the joint clinical assessment of the medicinal product Catequentinib. Updated dossier submission date: 9 April 2026.

The dossier includes information on two therapeutic indications for the treatment of cervical carcinoma and for the treatment of leiomyosarcoma, referred to as indication a and

15 Joint Clinical Assessments

4 Published

4 Discontinued

Requirements of Regulation (EU) 2021/2282	Section of the updated dossier	Missing information, data, analyses and other evidence
Article 9(2) Joint clinical assessment dossier “contains complete and up-to-date information, data, analyses and other evidence [...] to assess the parameters included in the assessment scope”	Section 4. Methods Section 5. Results	Rationale: According to Article 9(2) of Regulation (EU) 2021/2282 ⁽¹⁾ , the dossier shall contain complete and up-to-date information, data, analyses and other evidence submitted by the health technology developer to assess the parameters included in the assessment scope. Missing for indications a and b: - Transparent and comprehensive identification of all available evidence, followed by a transparent and thorough synthesis to assess the parameters defined in the assessment scope based on the totality of the evidence. - Justifications for the inclusion or exclusion of evidence, at a level which enables the assessment of whether the methodological approach is acceptable and, consequently, the validity of the results.
Article 9(2) Joint clinical assessment dossier “contains complete and up-to-date information, data, analyses and other evidence [...] to assess the parameters included in the assessment scope”	Section 3. Assessment scope	Rationale: According to the dossier template ⁽²⁾ , Section 3 shall: - reproduce the assessment scope in the format shared with the health technology developer in the Commission’s first request; - clearly identify any PICO(s), for which results were not submitted and explain the reasons for their omission. Missing for indications a and b: - Presentation of the PICOs in accordance with the Commission’s first request; - Explanation for the omission of results on relative effectiveness and relative safety of PICOs, which would allow the

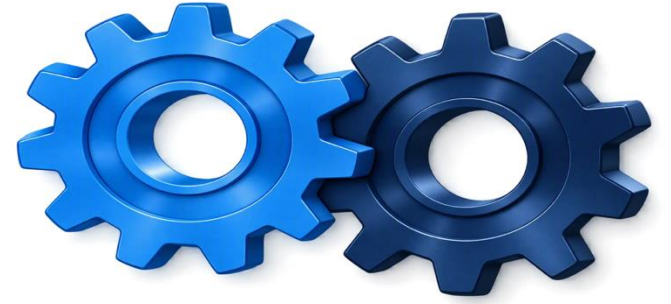
• 3 Insufficient data

Transparency

- Publication and national implementation?
- Factual accuracy or something else?

Faster publication means
streamlined national
implementation

Connectivity



- Evidence Scope
 - Landscaping the treatment pathways around Europe
 - Identifying the outcomes that matter at face of healthcare provision
 - Connecting evidence via indirect comparisons (and beyond?)
- Publication of relative effect metrics
 - A cross border, transparent record of relative effectiveness early
 - Follow up data can be published and we start to see longitudinal evidence
 - Connectivity to registry, potentially adaptive studies.
- Description of uncertainty and evidence gaps
 - Absence of evidence or absence of submission
 - Clear identification of the uncertainties can lead to better solutions

What should Evidence in the Real World look like?



Randomise as early as possible

Both sides prefer randomised evidence. Where classic RCTs are hard, use adaptive designs to preserve randomisation.



Use pragmatic & registry-based RCTs

Randomised trials run in routine care or built on disease registries can extend evidence beyond the tightly controlled pre-approval population.



Adopt a shared estimand framework

Regulators and HTAbs may need different estimands. Design trials that can estimate several in parallel, agreed with both audiences up front.

1 April 2025
EMA/115125/2025

Joint HTAb-regulatory perspectives on understanding evidence challenges, managing uncertainties and exploring potential solutions

Outcome of a workshop series between HTA bodies and regulators

What bodies are jointly recommending



Capture non-primary outcomes properly

Keep collecting disease status, symptoms, quality of life and safety after progression or switch to next therapy - don't stop at the primary endpoint.



Ensure timely access to patient-level data

IPD, including baseline covariates for both arms, is essential for robust indirect comparisons and confounding adjustment.



Use real-world data where it actually helps

- RWD is valuable for: natural history and comparator mapping.
- Post-hoc external comparators built after the fact remain unreliable for confirmatory decisions.

Novel designs are what make 'randomise early' realistic



Master protocols



**Seamless & platform
designs**



Bayesian adaptive designs



Investment in healthcare

How can HTA help
direct that
investment

Is evidence
generation just about
a single technology
or can it serve many
needs

09:35 – 09:45



Investing in life sciences

Kristin Thompson

Investment Director (Life Sciences),
Merieux Equity Partners

09:20 – 10:25

Navigating Europe's Future - Opportunities for RWE Innovation?



Hans-Georg Eichler

Moderator

Chief Consultant Physician, Association of Austrian Social Insurance Institutions

Slido

Ask your question



Roisin Adams

Head of HTA Strategy,
National Centre for
Pharmacoeconomics,
Ireland



Kristin Thompson

Investment Director
(Life Sciences), Merieux
Equity Partners



Alexander Natz

Secretary General,
EUCOPE



Niklas Hedberg

Chief Pharmacist, TLV



Wim van Harten

Research Group leader,
Netherlands Cancer
Institute

10:25 – 11:10

The European Health Data Space



Eric Sutherland *Moderator*

Senior Health Economist, OECD

Slido

Ask your question



Nienke Schutte

Head of Unit, Innovation in
Health Information Systems Unit,
Sciensano, Belgium



Christian Dehlendorff

Biostatistical Chief
Consultant, Danish
Medicines Council



Dorota Religa

MD, PhD, Director, SveDem,
Karolinska University
Hospital, Sweden



Diana Sinkevich

VP Market Access EU &
International and Global
HEOR, Chiesi

10:25 – 10:35



EHDS Implementation

Nienke Schutte

Head of Unit, Innovation in Health
Information Systems Unit, Sciensano,
Belgium

The AI future of evidence starts with the EHDS

dr Nienke Schutte

24.09.2026 RWE4Decisions symposium

EHDS infrastructure

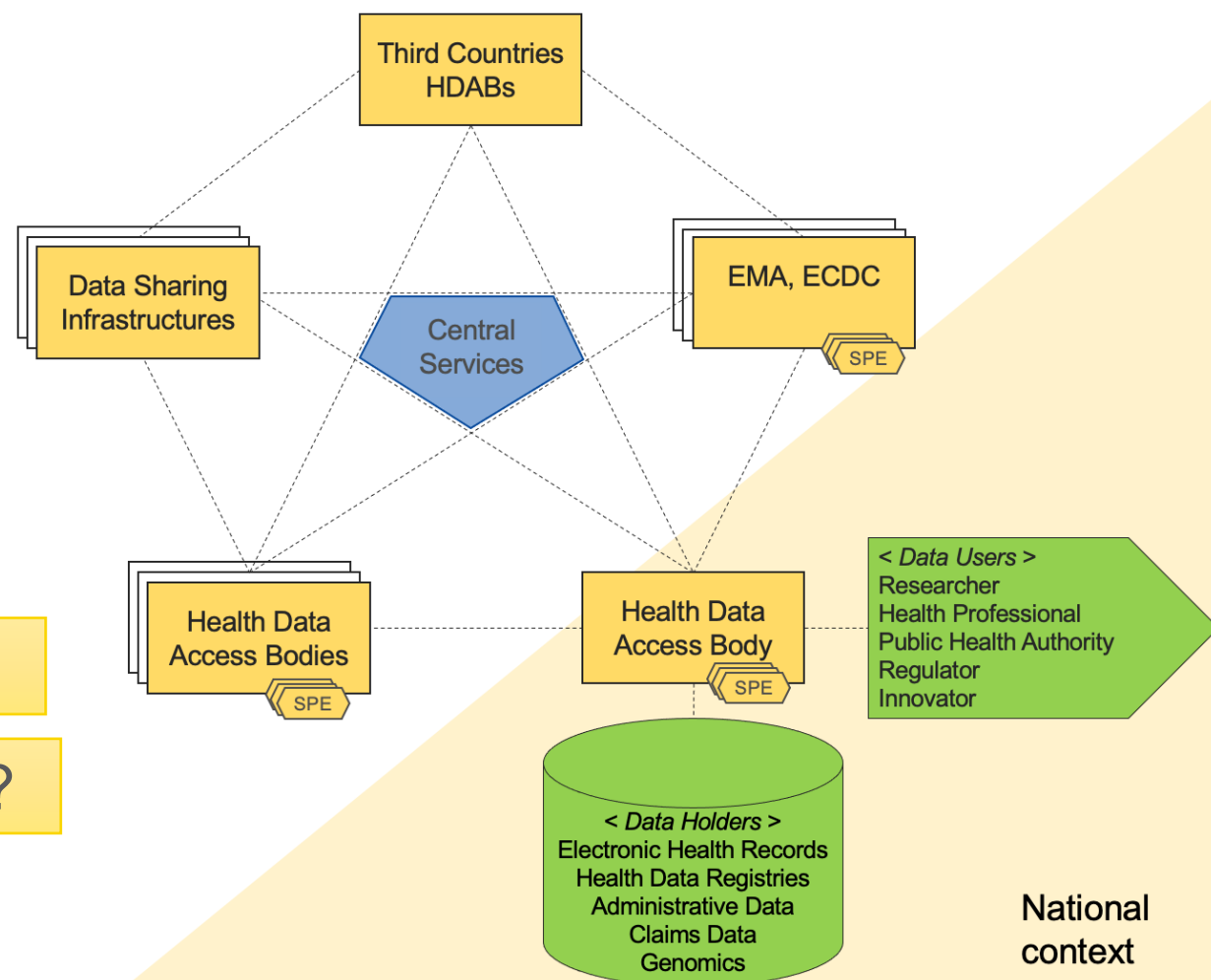
What are we building?

infrastructure for finding, accessing and (re)using health data across Europe

governance framework for health data

Does EHDS deliver for RWE today?

Is it ready for AI-enabled RWE tomorrow?



RWE requirements

Does EHDS meet the needs?

RWE requirements	EHDS contribution
Find right data	(EU) Dataset catalogue
Assess data	Data quality, utility, provenance
Access data	Permit acquisition through HDAB
Analyse data	Secure processing environments
Multicountry studies	HeathData@EU infrastructure

EHDS implementation

What are the challenges?

EHDS contribution

(EU) Dataset catalogue

Data quality, utility, provenance

Permit acquisition through HDAB

Secure processing environments

HeathData@EU infrastructure

Implementation requires...

Rich, machine-readable metadata; support and tools for data holders to create and maintain records

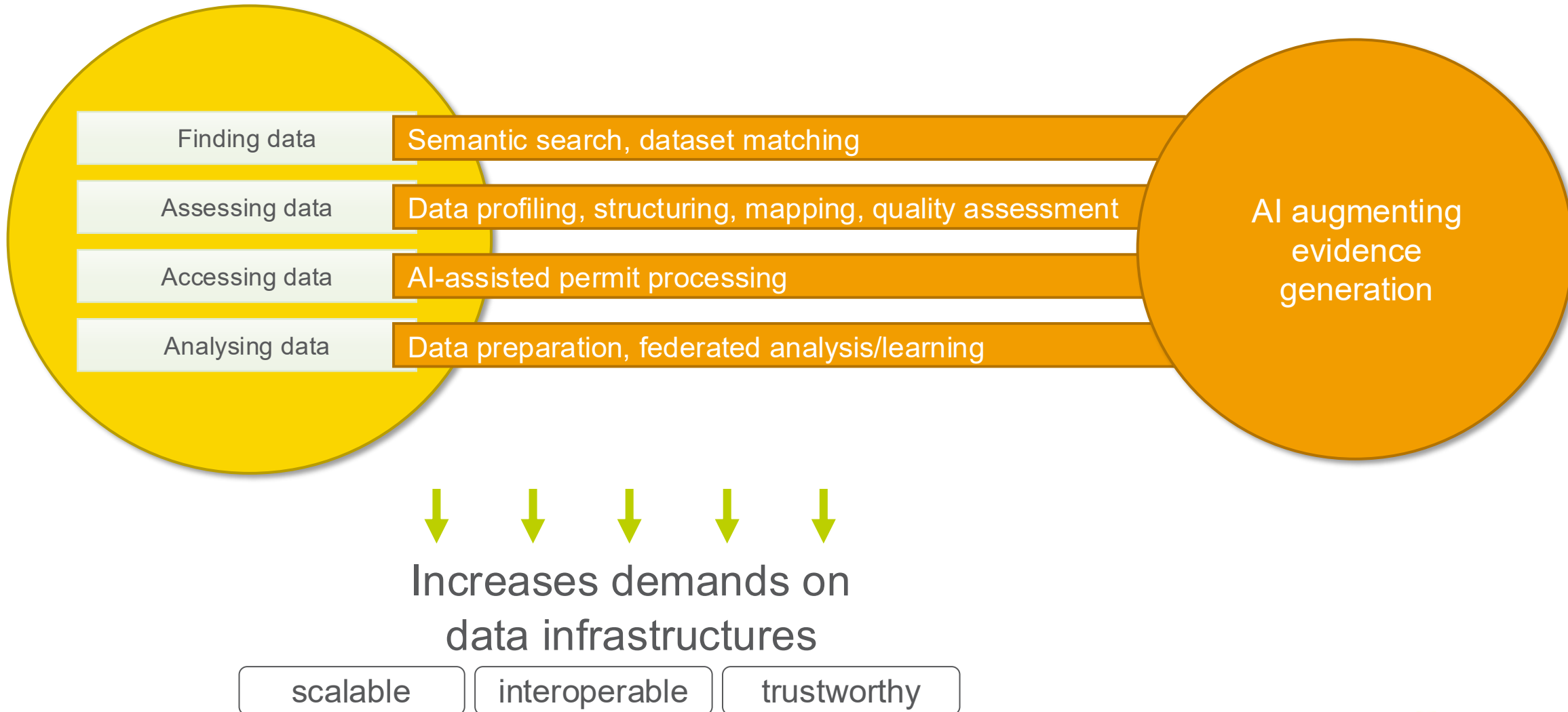
Tools and support for data holders to assess quality & improving data quality

Clear, harmonized and predictable application processes

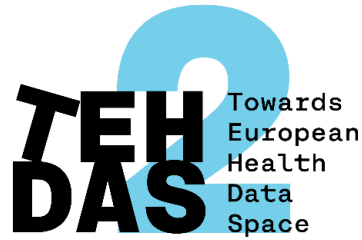
Flexible enough to support different types of health data and analytical needs, while maintaining strong security

Aligned cross-border processes, avoiding duplication and divergent workflows across HDABs

AI changes evidence generation

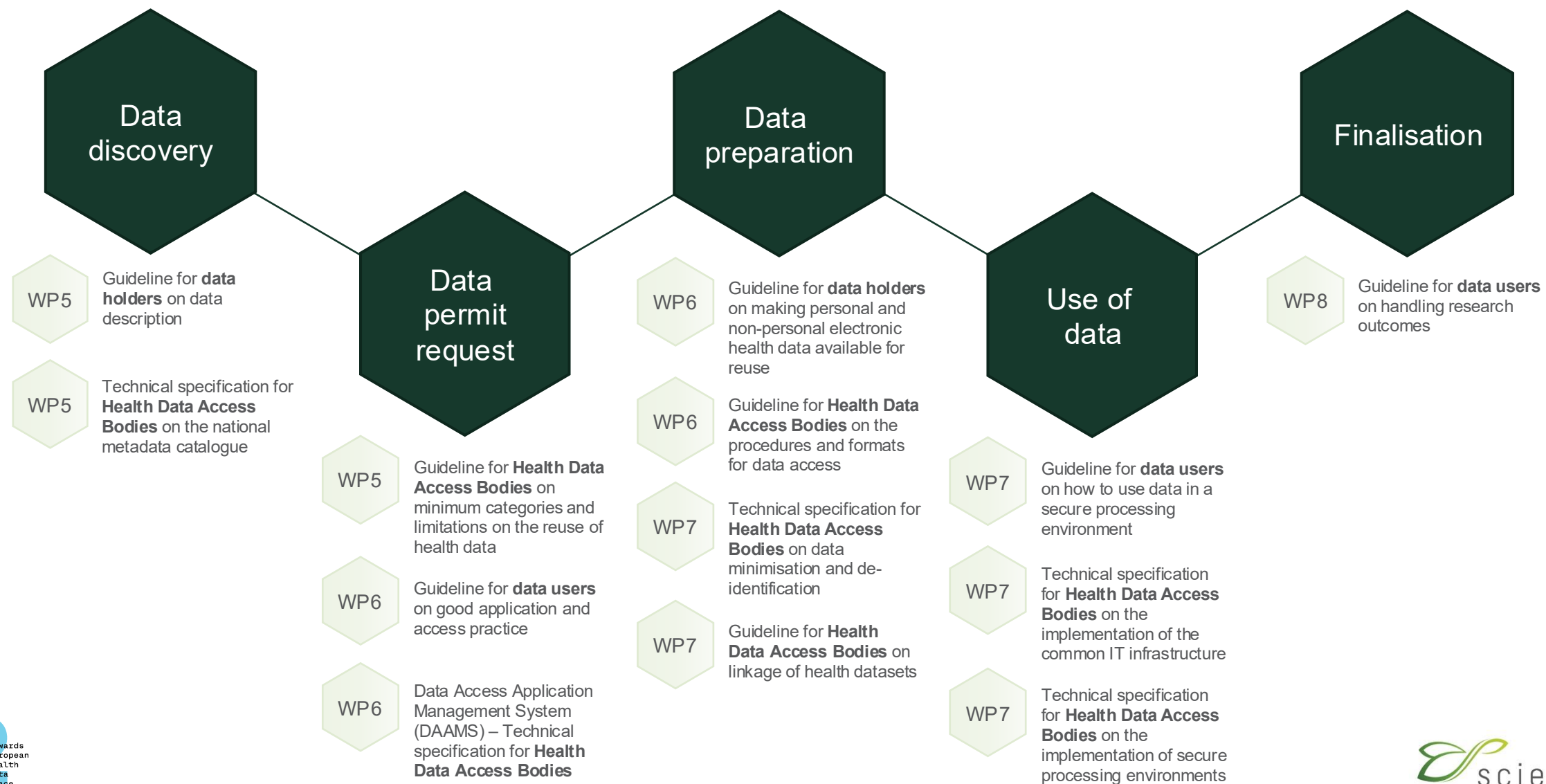


Harmonise the infrastructure



Develops common guidance, practical approaches and technical specifications to support harmonized implementation of the EHDS across Europe

Harmonise the infrastructure



Harmonise the infrastructure



WP4 Guideline for **Health Data Access Bodies** on fees and penalties for non-compliance regulated to the EHDS regulation

WP4 Guideline for **Health Data Access Bodies** on collaboration with **other parties**

WP4 Guideline for **Health Data Access Bodies** on international and third country access and transfer of electronic health data

WP5 Guideline for **Health Data Access Bodies** on enrichment of health datasets

WP8 Guideline for **Health Data Access Bodies** on data altruism in health

WP8 Guideline for **Health Data Access Bodies** on obligations towards natural persons

WP8 Guideline for **Health Data Access Bodies** on informing natural persons about the use of health data – "Citizen Information Point"

Harmonise the infrastructure

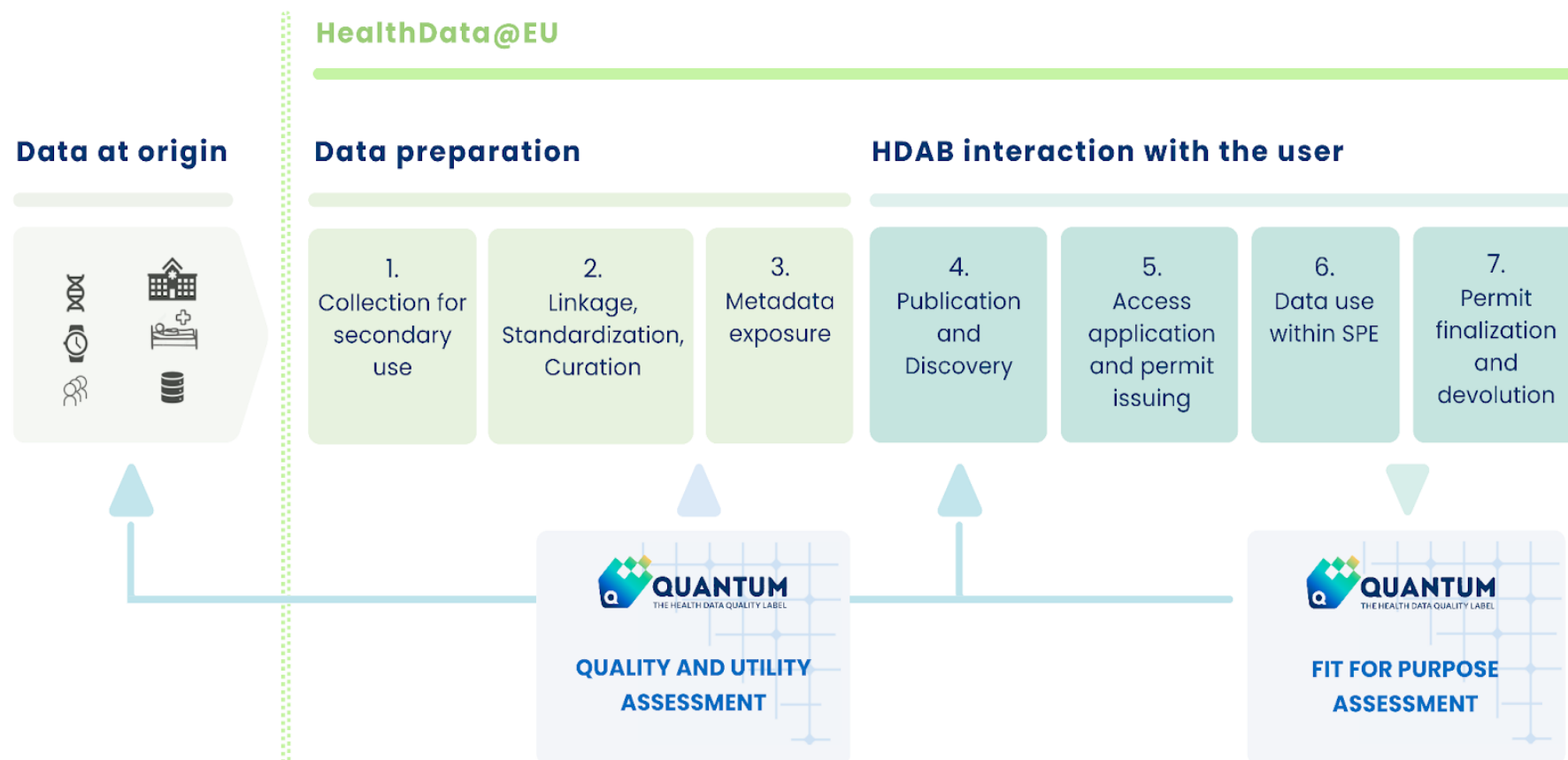


Develops common guidance, practical approaches and technical specifications to support harmonized implementation of the EHDS across Europe



Develops a common approach to describing data quality and utility in the EHDS, helping users assess whether data are fit for use and fit for purpose

Harmonise the infrastructure

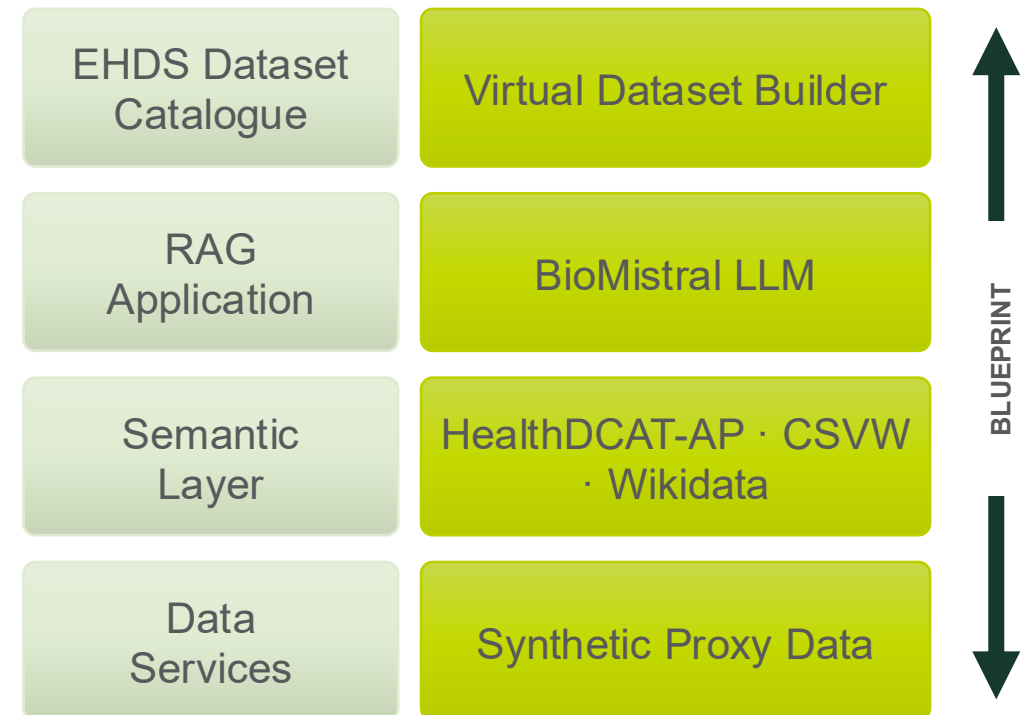


Augmenting the infrastructure

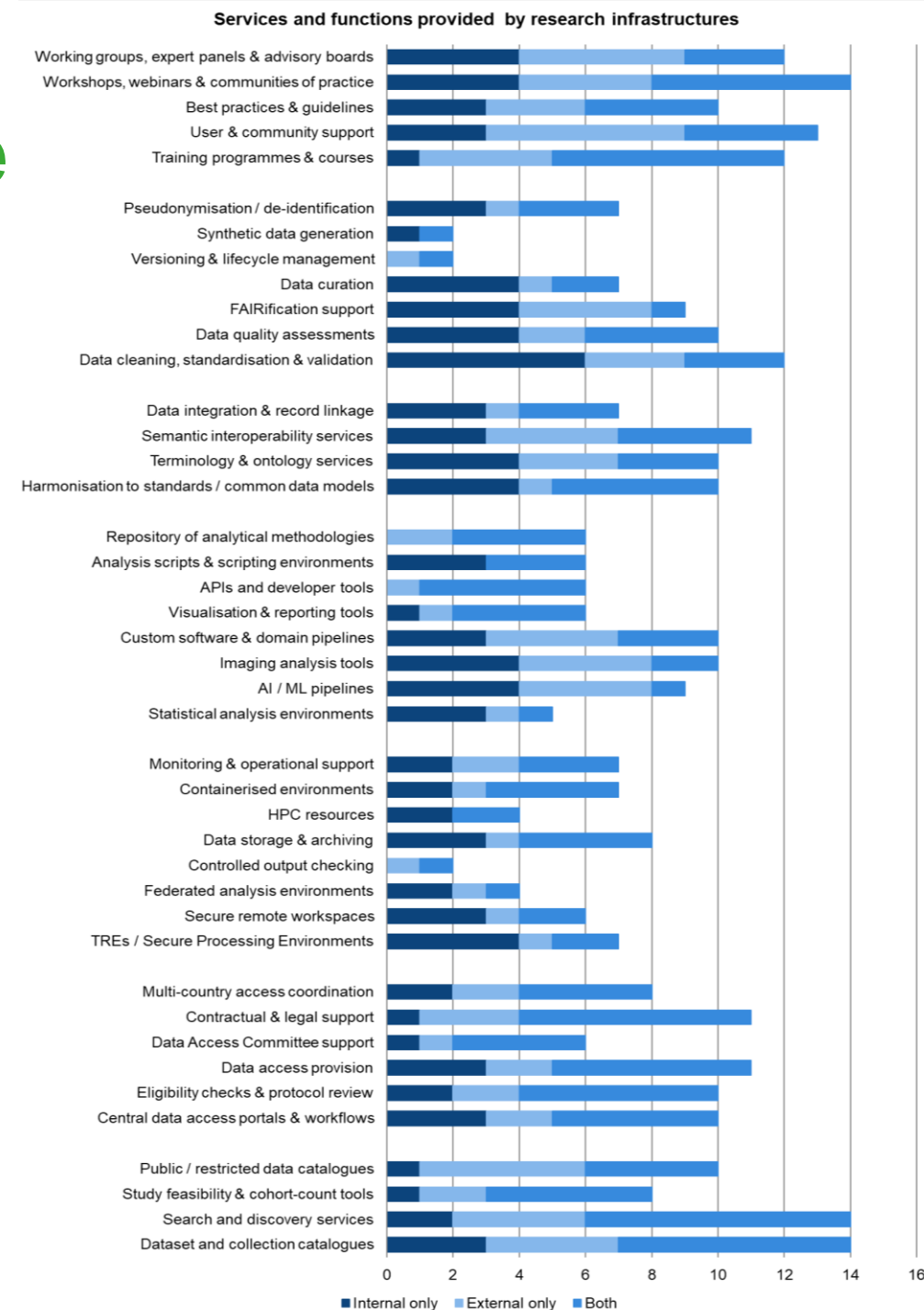
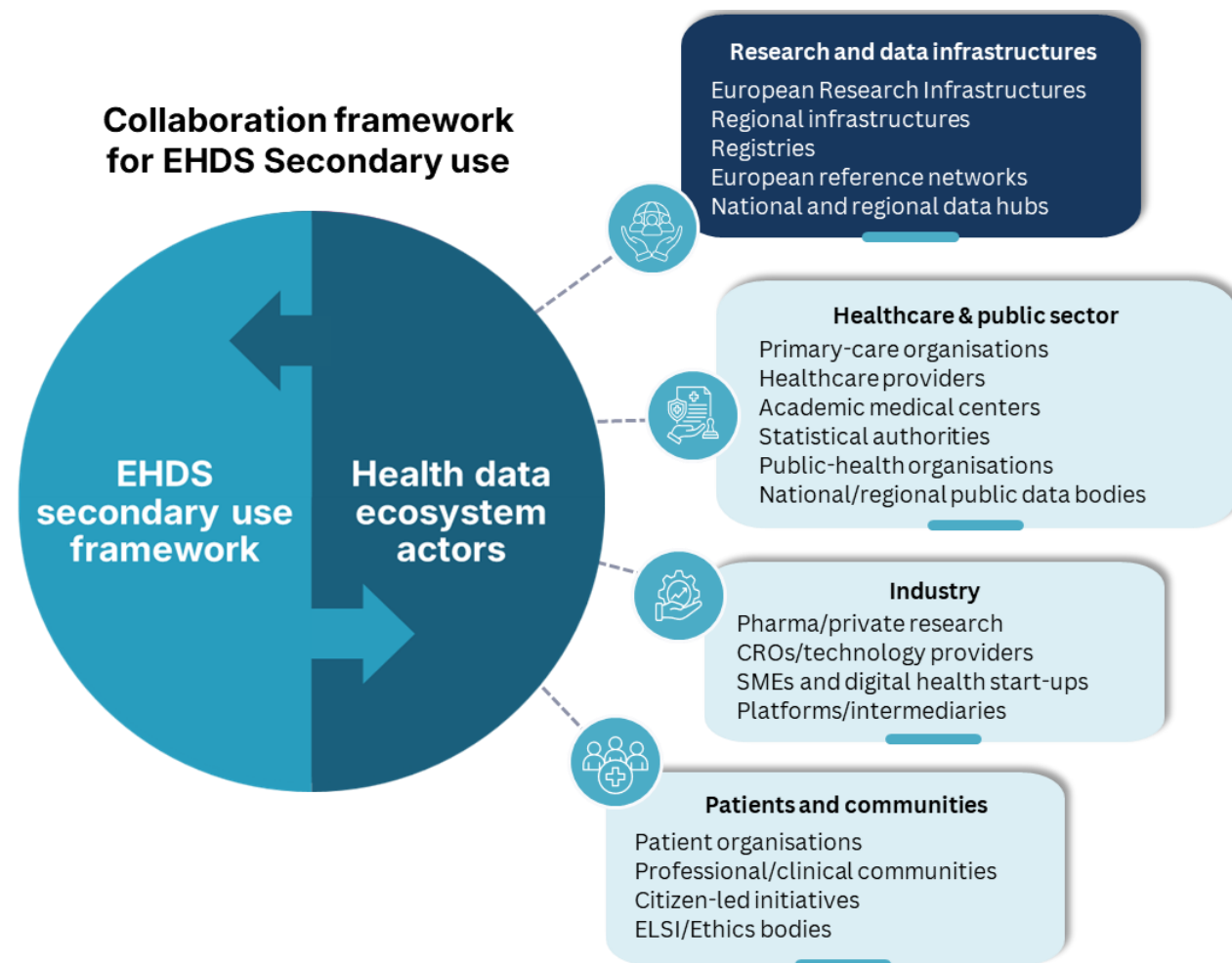


demonstrates how GenAI can enhance EHDS dataset catalogues:

For any research question, the system returns a virtual dataset, a mapping to real-world data assets, and proposed synthetic data



Connecting the infrastructure



Do we have the answers?

Does EHDS deliver for RWE today?

Yes, it is building the foundations. But a legal framework only creates value when it is translated into an operational, usable infrastructure

scalable

interoperable

trustworthy

Is it ready for AI-enabled RWE tomorrow?

It can be, if we align ecosystems, enhance the infrastructure, and connect it to the existing expertise and services

harmonise

augment

connect

dr. Nienke Schutte

Nienke.Schutte@sciensano.be

www.sciensano.be

10:25 – 11:10

The European Health Data Space



Eric Sutherland *Moderator*

Senior Health Economist, OECD

Slido

Ask your question



Nienke Schutte

Head of Unit, Innovation in
Health Information Systems Unit,
Sciensano, Belgium



Christian Dehlendorff

Biostatistical Chief
Consultant, Danish
Medicines Council



Dorota Religa

MD, PhD, Director, SveDem,
Karolinska University
Hospital, Sweden



Diana Sinkevich

VP Market Access EU &
International and Global
HEOR, Chiesi

11:10 – 11:40

Coffee break

See you in 30 minutes!



11:40 – 12:20

AI for RWE Generation



Shaun Rowark

Moderator

Associate Healthcare Director Data Analytics,
NICE

Slido

Ask your question



Jing Wang-Silvanto

Member, Observational Health
Data Sciences and
Informatics (OHDSI)



Saunya Dover

Director Science and
Methods, Canada's Drug
Agency (CDA-AMC)



Tonya Winders

President and Acting CEO,
Global Allergy & Airways
Patient Platform (GAAPP)

11:40 – 11:50



*A reasonable validation
framework for AI in RWE
generation?*

Shaun Rowark

Associate Healthcare Director Data Analytics,
NICE

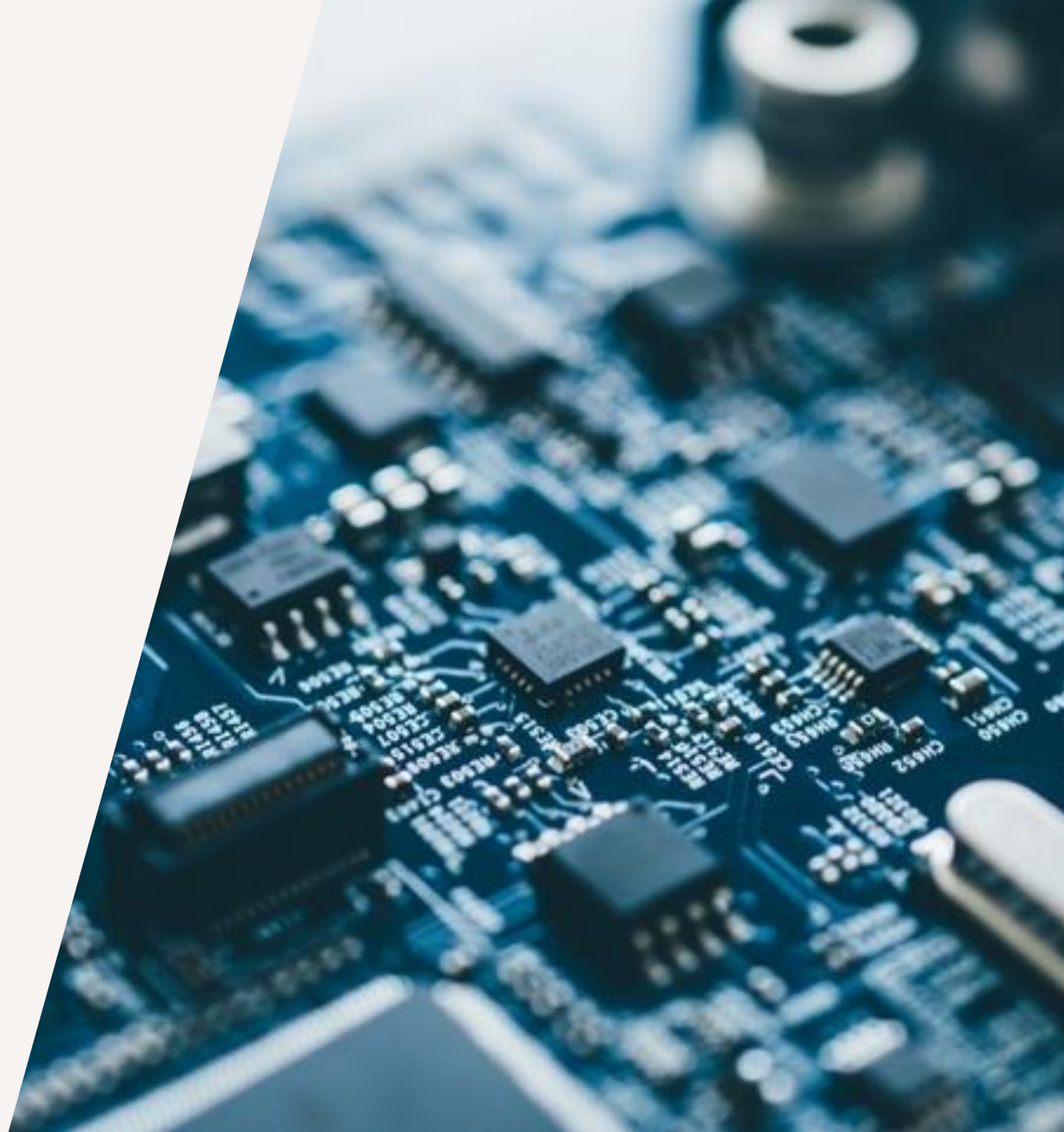
AI for RWE generation panel

Shaun Rowark

Associate Director, Healthcare Data Analytics

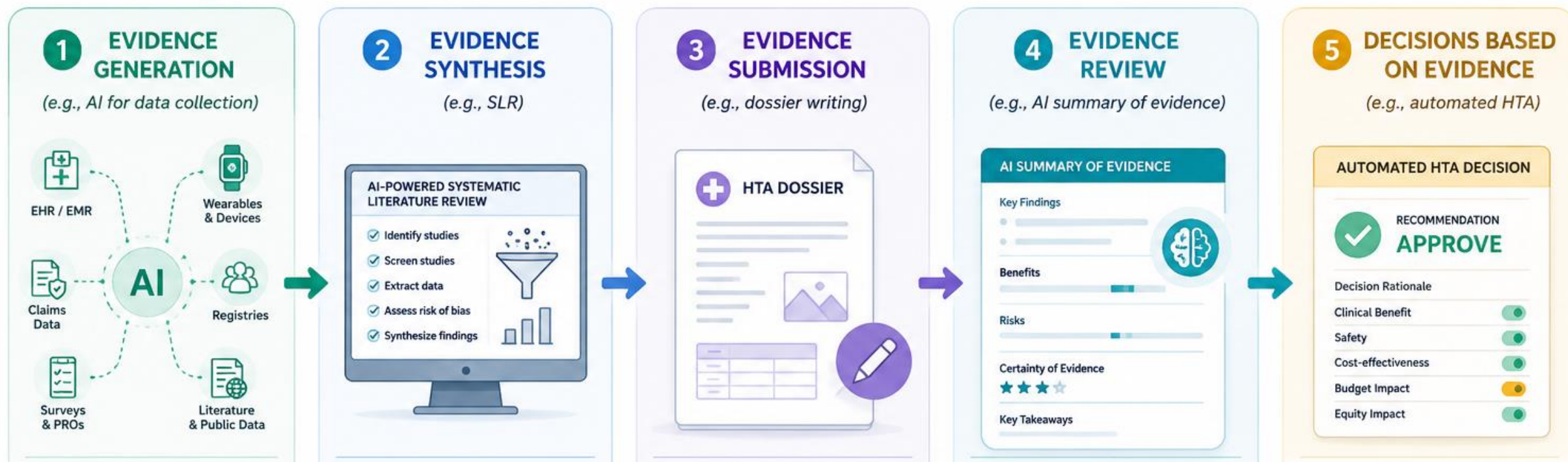
23 September 2026

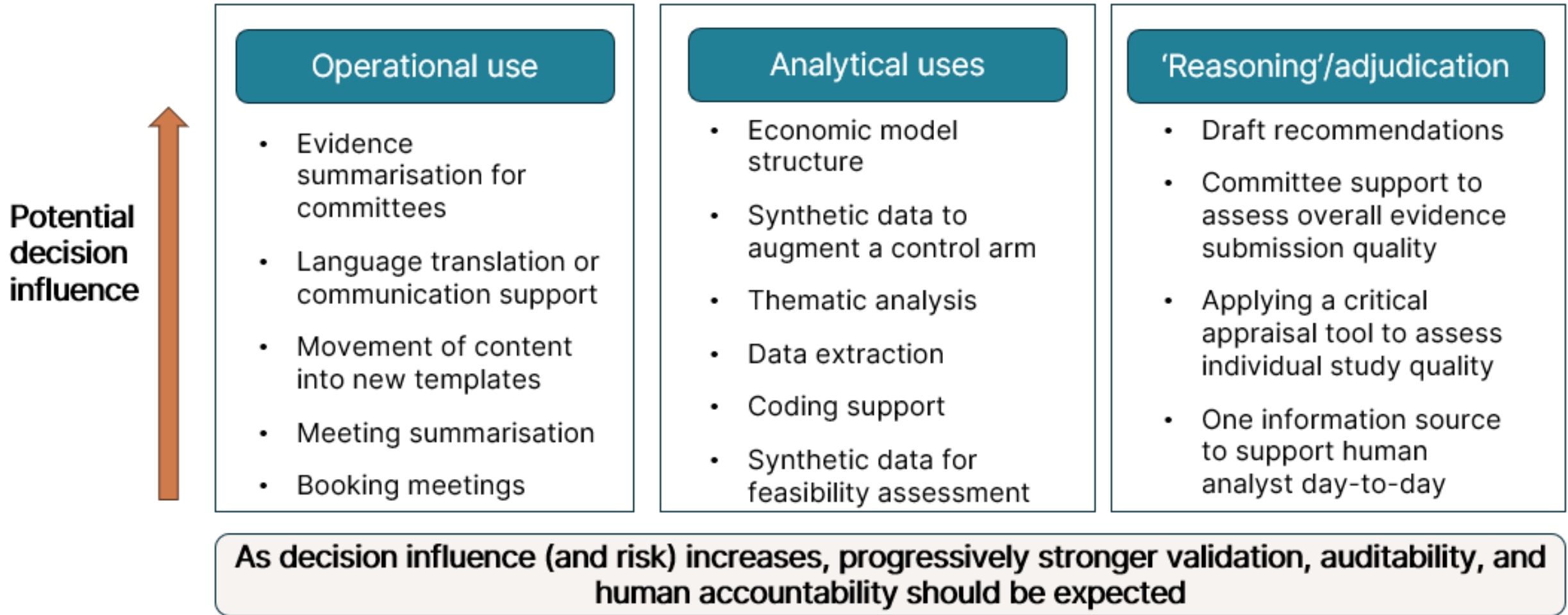
NICE National Institute for
Health and Care Excellence®



AI in the evidence lifecycle?

Disclaimer: Not NICE's views





*Wang-Silvanto et al (pre-publication)

NICE

Problem statement



Evidence
synthesis



Document
creation/review



Data
analytics



Economic
modelling



Causal
inference



Predictive
modelling

Direct implications on HTA, guideline development and decision making...

...AI brings...

Efficiency
Scalability
Novel approaches

...but with poor tools and lack of guidance...

Lack of transparency
Inconsistency across submissions
Downstream challenges for evaluation
Potential impact on trust and robustness

Scope

Principles-based framework with modular development aligned to evolving AI use cases.



In scope

- Cross-cutting principles
- Specific AI uses:
 - Primary evidence generation
 - Evidence synthesis
 - Economic modelling
 - Technical document preparation and review



Out of scope

- AI-enabled health technologies
- NICE business processes
- Appraisal of specific AI tools
- Regulation



Intended users

All involved, directly or indirectly, in an evidence submission to NICE.

Governance



External expert working group

- Rapid review of updates
- Participate in general surveys, targeted reviews, roundtables



Internal task & finish group

- Review surveys and analysis
- Ensure NICE operational alignment



Internal senior leadership steering group

- Resolve blockers
- Identify risk
- Ensure NICE strategic alignment

FOUNDATIONAL PRINCIPLES



Data compliance



Responsible use

GOVERNING THE USE OF AI



Accountability



Human oversight

ASSURING THE METHODOLOGY



Validation



AI lifecycle
management



AI SYSTEM



Fairness and
equity



Reproducibility

TRUSTING THE EVIDENCE



Explainability



Transparency

AI Declaration (AID) checklist



Purpose

Make AI use visible and reviewable

- Declare AI use across the submission.
- Show AI contributions, risk, biases, controls.
- Support interpretation, scrutiny, reproducibility and audit.
- Developed iteratively through the HTA Lab AI submission pilot.



Application

1-2

Map every use of AI

List all uses and determine influence.



When applicable

3-21

Detailed reporting

Explain and justify AI use, including inputs, outputs and human oversight.



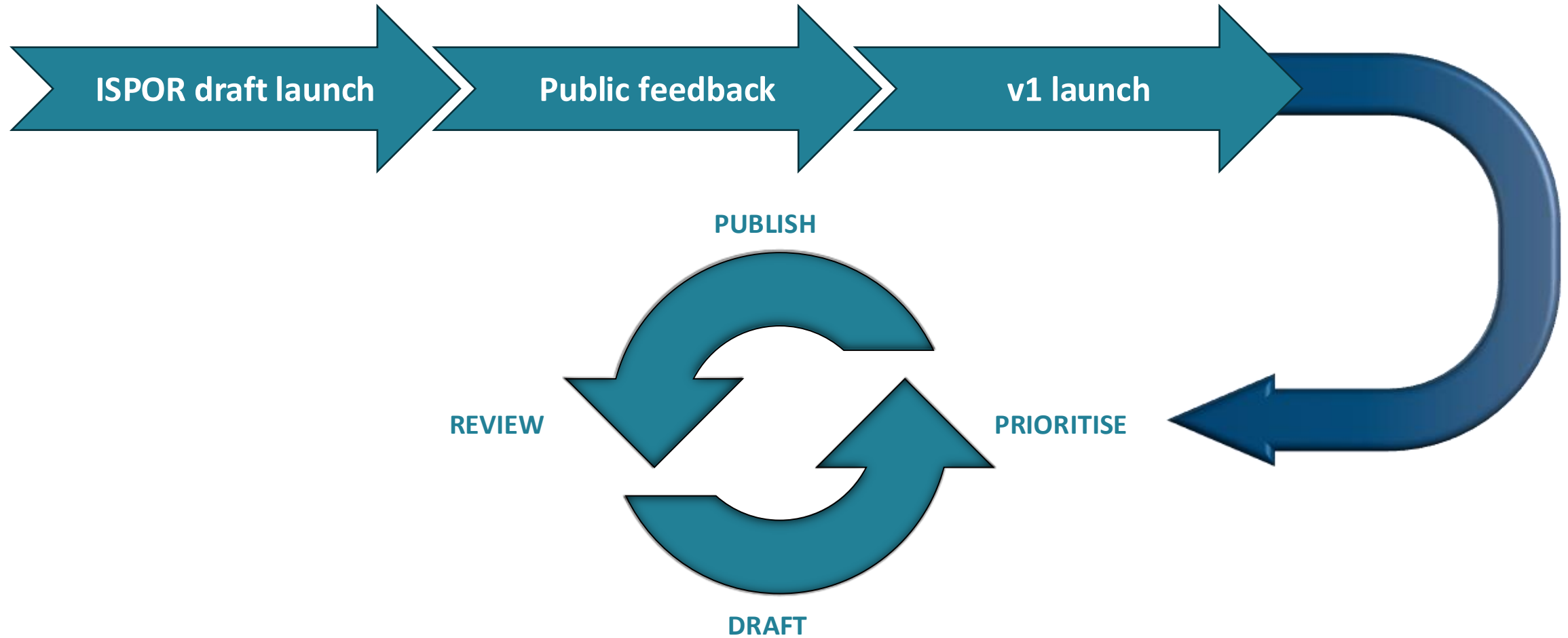
Alignment

Supports framework reporting

- Mirrors principles and terminology.
- Translates principles into reporting checks.
- Supports consistent, proportionate scrutiny across submissions and critiques.

Next steps

Agile updates



Thank you

ai@nice.org.uk

Stay up to date



- Sign up to our newsletters - scan the QR code or visit nice.org.uk/newsletters
- Follow us on social media - visit link.tr.ee/nicecomms

11:40 – 12:20

AI for RWE Generation



Shaun Rowark

Moderator

Associate Healthcare Director Data Analytics,
NICE

Find out more
about GAAPP's
AI tool to
support patients
living with COPD



Jing Wang-Silvanto

Member, Observational Health
Data Sciences and
Informatics (OHDSI)



Saunya Dover

Director Science and
Methods, Canada's Drug
Agency (CDA-AMC)



Tonya Winders

President and Acting CEO,
Global Allergy & Airways
Patient Platform (GAAPP)

11:40 – 12:20

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Ask your question



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12:20 – 12:55

RWE4Decisions Priorities to Support Better RWE Generation for HTA/ Payer Decision-Making



Karen Facey

Moderator

Senior HTA Adviser, RWE4Decisions Secretariat;
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Ask your question



Hans-Georg Eichler

Chief Consultant Physician,
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Klaus Pugner

Head of Global Payer
Evidence Planning and
HTA, Takeda

12:20 – 12:30



Review of RWE4Decisions' 2026 activities

Karen Facey

Senior HTA Adviser, RWE4Decisions
University of Edinburgh

Rare Diseases: Improving RWD Quality & Availability for HTA/Payers

Webinar | 8 April 2026



WEBINAR REPORT

Improving Rare Disease Real-World Data Quality and Availability for HTA/Payers

Wednesday, 8 April 2026, 15:00–16:30 CEST

Keynote speaker

PROF. MAURIZIO SCARPA, MD, PhD
Coordinator, European Reference Network for Rare Hereditary Metabolic Diseases (MetabERN)

Speakers

PROF. DR. IR. WIM GOETTSCHE
Special Advisor HTA, ZIN

DR. NICOLE MITTMANN, PhD
Chief Scientist and Vice-President of Evidence, Canada's Drug Agency (CDA-AMC)

FRANÇOIS HOUÏEZ
Information & Access to Therapies Director, Health Policy Advisor, EURORDIS

ALESSIO AMADASI
Vice-President Medical Affairs EU/International, Rare Diseases, Chiesi Group

Co-Moderators

VICTORIA HODGKINSON
Executive Director, Canadian Neuromuscular Disease Registry

KAREN FACEY
RWE4Decisions Facilitator

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- Limited clinical data leads to major evidence uncertainties
- International registries provide long-term follow-up of individual patients to characterise disease, identify unmet needs, evaluate patient outcomes (including quality of life) after intervention
- RWD needs to be reliable, interoperable, decision-relevant to support use in patient care and decision-making
- Early collaboration and investment is needed to ensure that relevant high-quality data are collected for various different decision-making settings for care of people living with rare diseases

Rare Diseases: Improving RWD Quality & Availability for HTA/Payers

Webinar | 8 April 2026



- ! Address data collection burden
 - Collaborate early to identify core outcomes
 - Use digital technologies for data capture?
 - Provide incentives for good quality data collection (funding and feedback to clinical teams)
 - Explore patient experience of data collection requirements
- ! Feedback to
 - Clinicians to improve data quality and inform patient care
 - Patients to indicate how their data are used

Can Real-World Data Explain Patient Benefit?

Roundtable | 3 June 2026

RWE4Decisions

3 June 2026

Can Real-World Data Explain Patient Benefit?

Report of Roundtable – 3 June 2026

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Honing patient-relevant outcomes for real-world use – Steven Harjes (Hematon)	5
Q&A	6
Breakout group discussions	7
Exclusive insights from HTA representatives	8
Conclusion	10

Introduction

On 3 June 2026, the RWE4Decisions multi-stakeholder learning network organised a virtual roundtable meeting entitled “Can Real World Data Explain Patient Benefit?”. It brought together 65 participants from across the healthcare landscape in Europe and North America, including staff from health technology assessment (HTA) bodies, public payers, national health ministries, regulatory agencies, EU institutions the pharmaceutical industry and patient groups; data analysts, registry holders and academics. The meeting enabled rich, cross-sector discussion about how patient relevant outcomes could be captured in the real-world setting.

Co-moderated by Eric Sutherland (Organisation for Economic Cooperation and Development (OECD)) and Karen Facey (RWE4Decisions Senior HTA Advisor), the meeting opened with a scene-setting intervention, followed by a topic introduction and three case study presentations that grounded the discussion in practical experience about development of patient relevant outcomes for use in the real-world setting. These inputs shaped dynamic exchanges during the multistakeholder breakout discussions that fed into the plenary reflections. Notably, this Roundtable marked the first edition to feature dedicated perspectives from HTA/Payers, adding valuable decision-maker insights and strengthening the overall depth of the discussion.

- HTA determination of added benefit focuses on “patient-relevant” outcomes in clinical trials, but what about in real-world?
- Patient benefit can be measured by PROMs, which have clear process for validation, used by industry, clinicians, patient groups
- PROMs can be integrated into registries and combined with other RWD
- Data quality challenges exist with use of PROMs as evidence in HTA (even in clinical trials) – could digital tools help?

effectively.

Can Real-World Data Explain Patient Benefit?

Roundtable | 3 June 2026

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Can Real-World Data Explain Patient Benefit?

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- Are the PROs really patient-relevant?
? Could data collection be real-world responsive, based on change in condition/experience of side effects (particularly in cyclical treatment)
- Think beyond PROMs that measure quality of life to establishment of core outcome sets (PROBE study)
- Data collection should create value for patients and clinicians, in addition to developing a robust evidence set

effectively.

Alzheimer's Disease: Planning RWD Collection to Inform HTA

Roundtable | 18 February 2026

RWE4Decisions

18 February 2026

Planning Real-World Data Collection to Inform HTA Alzheimer's Disease

Report of Roundtable - 18 February 2026

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The role of NICE in the ConnectD project informing development of a national AD registry: Shaun Rowark (NICE)	4
How can RWE complement RCTs to demonstrate the burden of Alzheimer's Disease and benefits of treatment to patients and carers?: Matt Dixon (Bristol Myers Squibb)	4
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On the 18th of February 2026, the RWE4Decisions multi-stakeholder Learning Network hosted a Roundtable virtual meeting on **Planning real-world data (RWD) collection to demonstrate real-life effectiveness and inform economic modelling, using Alzheimer's disease (AD) as a case study**. Over sixty participants representing HTA/Payers, national health ministries, regulators, EU institutions, patients, data analysts, registry holders, academics, and the pharmaceutical industry participated. The meeting was co-moderated by Niklas Hedberg (TLV) and Karen Facey (RWE4Decisions Senior Advisor). A keynote presentation and three case study presentations, was followed by panel questions, multi-stakeholder breakout discussions and plenary reflections.

- Over 100 medicines in development
- To treat specific aspects of dementia – substantial patient and carer burden – large RCTs possible
- Pre-diagnosis “mild cognitive impairment”
 - Potentially large population – hard to identify
 - Patient relevant effects will be longer-term and difficult to measure
- RWD could support identification of relevant population for health system planning, when to initiate treatment, dose adjustment, discontinuation etc

effectively.

Alzheimer's Disease: Planning RWD Collection to Inform HTA

Roundtable | 18 February 2026

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- RWD could address wider aspects such as self-care, behavioural stability, crisis episodes, admission to hospital or specialist care
- Requires data collection across primary, social and specialist care
- Minimize burden on patients and carers (who are generally older)
- Given slow progression of disease, collect data annually
- Limit core dataset with addition of items, only if another taken out
- Include medicine modules in registries

Horizon Scanning to Support Early Dialogues & RWE Generation?

Webinar | 15 July 2026

A graphic representing a webinar report. It has a blue header with a DNA helix pattern. The text 'WEBINAR REPORT' is on the left, and the 'RWE4Decisions' logo is on the right. Below the header, the title 'How Can Horizon Scanning Support Early Dialogues and RWE Generation?' is displayed, followed by the date and time 'Wednesday, 15 July 2026, 15:00-16:30 CEST'. The main body lists the keynote speaker, speakers, and co-moderators with their names, titles, and photos. At the bottom, the website 'rwe4decisions.com' and the hashtag '#RWE4Decisions' are shown.

WEBINAR REPORT **RWE4Decisions**

How Can Horizon Scanning Support Early Dialogues and RWE Generation?

Wednesday, 15 July 2026, 15:00-16:30 CEST

Keynote speaker

MARCUS GUARDIAN
General Manager, International Horizon Scanning Initiative (IHSI)

Speakers

KIM HELLEBERG MADSEN
Chair EHT Subgroup, Director of Pharmacoeconomics & Availability Department, Danish Medicines Agency

LARA WOLFSON
Associate Vice President & HTA Statistics, MSD

BJÖRN WETTERMARK
Professor, Department of Pharmacy, Drug Epidemiology, Uppsala University, More-EUROPA project

Co-Moderators

MATIAS OLSEN
Associate Director, Public Affairs & Policy, EUCOPE

KAREN FACEY
RWE4Decisions Senior Advisor HTA

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- IHSI supports Payers to plan for products coming down the pipeline and predict their reimbursement indication
- It could be used to identify
 - Products with evidence risks (decision-relevant evidence gaps) and support collaborative planning and early conduct of RWE studies
 - Need for additional RWD collection in existing sources (e.g. novel endpoints, genetic testing, etc.)
 - Classes of products where new RWD collection mechanisms need to be organised

Demonstrating transportability of real-world evidence: what do HTA bodies and health technology developers need?

Ashley A. Jaksa¹, Blythe Adamson², Robert Szulkin³, Christian Dehlendorff⁴, Emma Hernlund³, Vandana Ayyar Gupta⁵, Melinda Hanisch⁶, Jing Wang-Silvanto⁷ and Karen M. Facey^{8,9}

¹Pedestal Health, USA; ²University of Washington, USA; ³Dental and Pharmaceutical Benefits Agency, Sweden; ⁴Danish Medicines Council, Denmark; ⁵National Institute for Health and Care Excellence, UK; ⁶Merck & Co Inc, USA; ⁷Astellas Pharmaceutical A/S, Finland; ⁸RWE4Decisions Secretariat, FIPRA, Belgium and ⁹University of Edinburgh, United Kingdom

Abstract

Real-world evidence (RWE) is reshaping how health technology assessment (HTA) bodies evaluate therapies, yet a critical gap persists: while transportability methods enable researchers to adapt treatment effect estimates from RWE studies in another jurisdiction to local contexts, the field remains unclear on when these analyses impact decision-making. This article synthesizes insights from HTA bodies, health technology developers (HTDs), and methodologists to clarify what is missing from today's transportability conversation. We find that methodological progress has outpaced guidance on its utility; HTDs risk investing in transportability analyses without clear evidence that they reduce key uncertainties or affect recommendations. We identify three fundamental gaps: the absence of objective thresholds for determining whether transportability assumptions are adequately met; limited guidance on when quantitative transportability is needed versus qualitative assessment; and variation in HTA bodies' evaluation and application of transported evidence. These gaps create a critical impasse where HTDs must decide whether to invest in such analyses, while HTA bodies lack standardized frameworks for their assessment. We argue that transportability cannot transition from academic exercise to a decision-making tool without explicit early dialogue between stakeholders, transparent reporting standards, and capacity building within HTA bodies. We present actionable recommendations to build HTA expertise, create a case study repository, and define contexts where transported RWE demonstrably impacts patient access. As evidence dossiers increasingly rely on nonlocal data, addressing these strategic and operational gaps becomes essential to help patients.

A strategic view of transportability

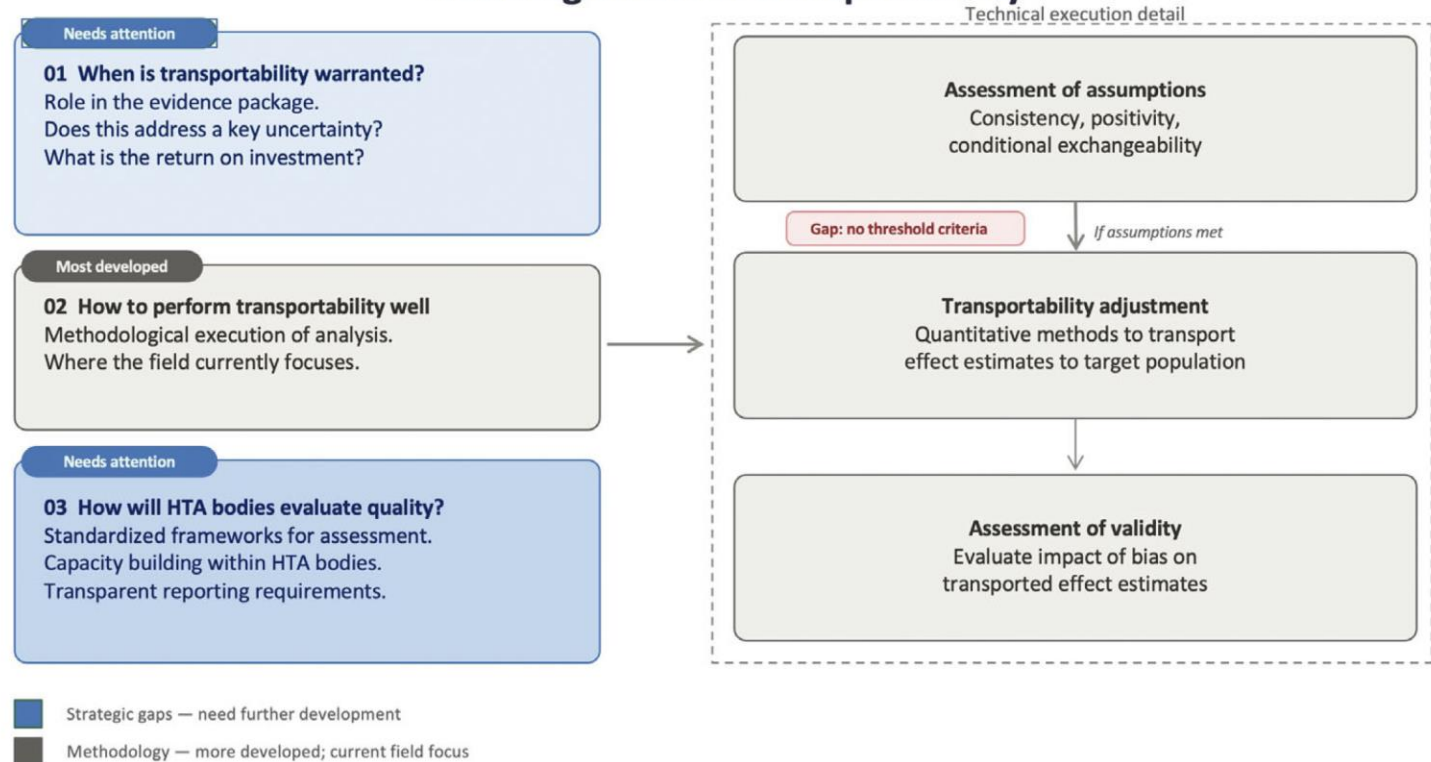


Figure 1. Pivoting the focus from transportability methods to their strategic use.

12:20 – 12:55

RWE4Decisions Priorities to Support Better RWE Generation for HTA/ Payer Decision-Making



Karen Facey *Moderator*

Senior HTA Adviser, RWE4Decisions Secretariat;
University of Edinburgh

Slido

Ask your question



Hans-Georg Eichler

Chief Consultant Physician,
Association of Austrian
Social Insurance Institutions



Ashley Jaksa

Principal for Research
Partnerships,
Pedestal Health



François Houÿez

Director of Treatment
Information and Access,
EURORDIS



Klaus Pugner

Head of Global Payer
Evidence Planning and
HTA, Takeda



Existing or non-existing data sources to be ALWAYS discussed during SA – JSC, EHDS to accelerate access to data (not 2 years!)



Developers should create real-world data when they don't exist, and not rely on published evidence (often incomplete or unfit)



Contradictions between RCT and RWD. Which control data truly support a comparison? We don't understand everything



Patients in the governance of data sources! Even for sources of data that don't see patients

For 2027



Larger scale training / upgrading of data analysts; (SMEs) struggle to perform high-quality rigorous analysis



Standardisation +++, particularly from public data sources (heterogeneity between different hospitals)

Thank you for your attention



François Houyez

francois.houyez@eurordis.org

12:20 – 12:55

RWE4Decisions Priorities to Support Better RWE Generation for HTA/ Payer Decision-Making



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Head of Global Payer
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HTA, Takeda

12:55 – 13:00



Closing remarks

Laura Batchelor

Managing Partner, FIPRA International

RWE4Decisions Learning Network co-creation: Stakeholder Actions to Generate Better RWE for HTA/Payers



Newsletters

Stakeholder Actions to Generate Better Real-World Evidence for HTA/Payers

The RWE4Decisions Learning Network has developed a new set of Stakeholder Actions to support stakeholders across the ecosystem in better RWE generation and use that respond to HTA/Payer needs.

We want to hear from you!

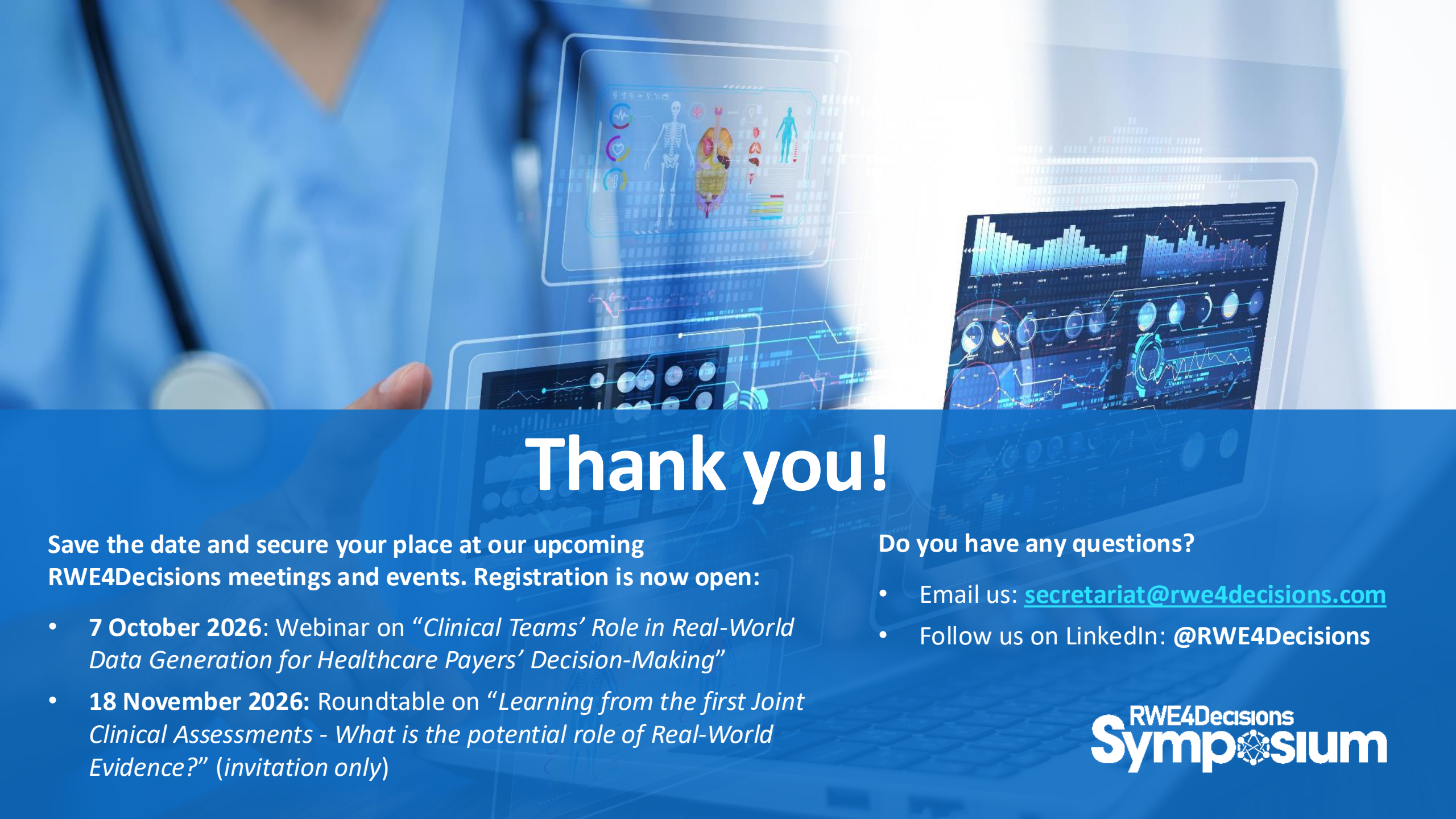
[Share your implementation story >](#)

ISPOR
Improving healthcare decisions

**New Actions launched at
RWE4Decisions Annual Symposium
and ISPOR RWE Summit**
New paper [publication](#) July 2025 (IJTAHC)

Now: Get Involved!

**Share your implementation story
on the RWE4Decisions website!**



Thank you!

Save the date and secure your place at our upcoming RWE4Decisions meetings and events. Registration is now open:

- **7 October 2026:** Webinar on *“Clinical Teams’ Role in Real-World Data Generation for Healthcare Payers’ Decision-Making”*
- **18 November 2026:** Roundtable on *“Learning from the first Joint Clinical Assessments - What is the potential role of Real-World Evidence?”* (invitation only)

Do you have any questions?

- Email us: secretariat@rwe4decisions.com
- Follow us on LinkedIn: [@RWE4Decisions](#)

RWE4Decisions
Symposium