REAL WORLD EVIDENCE
RWE4Decisions Symposium

Developing Real-World Evidence to Deliver Innovation in HTA

14 November 2024

BIP Meeting Center, Brussels

www.rwe4decisions.com

#RWE4Decisions

09:25 CET

Welcome

Laura Batchelor

RWE4Decisions Secretariat
FIPRA International

RWE4Decisions

HTA/Payer-led, multi-stakeholder Learning Network

RWE4Decisions brings together experts from all stakeholder groups to engage in dialogues that consider how fit-for-purpose RWE can be generated over the life cycle of **highly innovative medicines** through:

- 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 is generated by HTDs and will be assessed by HTA and used by Payers/decision-makers
- aligning planning and execution of effective Post Launch Evidence Generation (PLEG) studies.

RWE4Decisions REAL WORLD EVIDENCE 2024 STEERING GROUP

HTA bodies / Payers

Jo De Cock

Senior Adviser, **INAMI-RIZIV**

Diane Kleinermans

President. of Drug Reimbursement Commission, **INAMI-RIZIV**

Niklas Hedberg

Chief Pharmacist, **TLV**

Piia Rannanheimo

Chief Specialist, **Fimea**

Cláudia Furtado

Head HTA, P&R Div. and Information & Strategic Planning, **INFARMED**

Laurie Lambert

Special Projects Adviser, **CADTH**

Carlos M. Saborido

Director of the HTA Agency, **AETS**

International Org.

Eric Sutherland

Senior Health Economist, **OECD**

Industry

EUCOPE

AstraZeneca

Boehringer Ingelheim

GILEAD

MSD

NOVARTIS

Pfizer

Roche

Takeda

Patient Representatives

Chris Sotirelis

Patient Advocate for **Thalassemia**

EURORDIS RARE DISEASES EUROPE

Insurer

Hans-Georg Eichler

Consulting physician, **Austrian Social Insurance Inst.**

Clinician

Matti Aapro

Director, **Genolier Cancer Centre**

Analytics Expert

Ashley Jaksa

Market Access Scientific Strategy Lead, **Aetion, US**

Academia

Entela Xoxi

Pharmacologist, **Uni. Cattolica Sacro Cuore**

Facilitators

FIPRA International

Karen Facey, Senior Adviser (HTA)

François Meyer, Special Adviser (HTA)

Secretariat provided by FIPRA funded by EUCOPE and member companies

Housekeeping rules

This event is being livestreamed

The recording will be available on the RWE4Decisions website

If you are watching online, you can use the chat to interact with other viewers

To ask a question to the panel, raise your hand or type it in Slido.com specifying which speaker the question is addressed to

Don't forget to mention your name and affiliation when asking a question

We encourage you to support @RWE4Decisions via LinkedIn using #RWE4Decisions when sharing content about the Symposium.

09:30 CET

Plenary Session: The Policy Context for Real-World Evidence to Support Innovation in HTA

Moderated by

François Meyer

RWE4Decisions Facilitator
FIPRA Special Advisor HTA

09:35 CET

Plenary Session: The Policy Context for Real-World Evidence to Support Innovation in HTA

Moderated by Dr François Meyer, RWE4Decisions Facilitator

Council of the EU perspective

Enrique Terol García

Coordinating Advisor on Health, Permanent Representation of Spain to the European Union

09:45 CET

Opening Session

Real-World Evidence in the 2024-2025 EU health agenda

MEP Tomislav Sokol

Member of the European Parliament's Committee on the Environment, Public Health and Food Safety (ENVI)

10:00 CET

Plenary Session: The Policy Context for Real-World Evidence to Support Innovation in HTA

Moderated by Dr François Meyer, RWE4Decisions Facilitator

Regulator perspective

Patrice Verpillat

Head of Real-World Evidence,
European Medicines Agency (EMA)

EUROPEAN MEDICINES AGENCY
SCIENCE MEDICINES HEALTH

RWE update & EMANS 2028

RWE4Decision Symposium

14 November 2024 | Brussels, Belgium

Patrice Verpillat

Head of Real-World Evidence, Data Analytics & Methods Task Force

Disclaimer

I have no conflict of interests

The views expressed in this presentation are mine and should not be understood or quoted as being made on behalf of or reflecting the position of EMA or one of its committees or working parties

REMINDER

3 main pathways to use RWD for generating RWE @EMA

EMA studies using in-house databases

- **Primary and secondary care** health records from **France**, **Germany and UK**

Studies procured through EMA FWCs

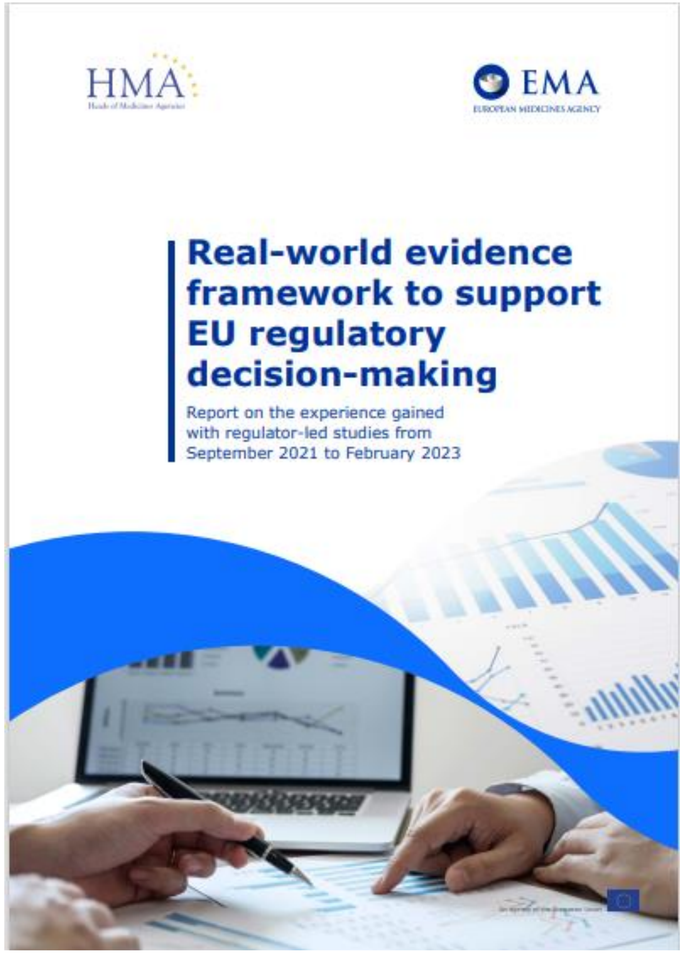
- Framework contract (FWC) since September 2021: services of **8 research organisations** and academic institutions
- Access to **wide network of data sources**: 59 data sources from 21 EU countries
- Ability to leverage external **scientific expertise**

DARWIN EU

DARWIN EU®

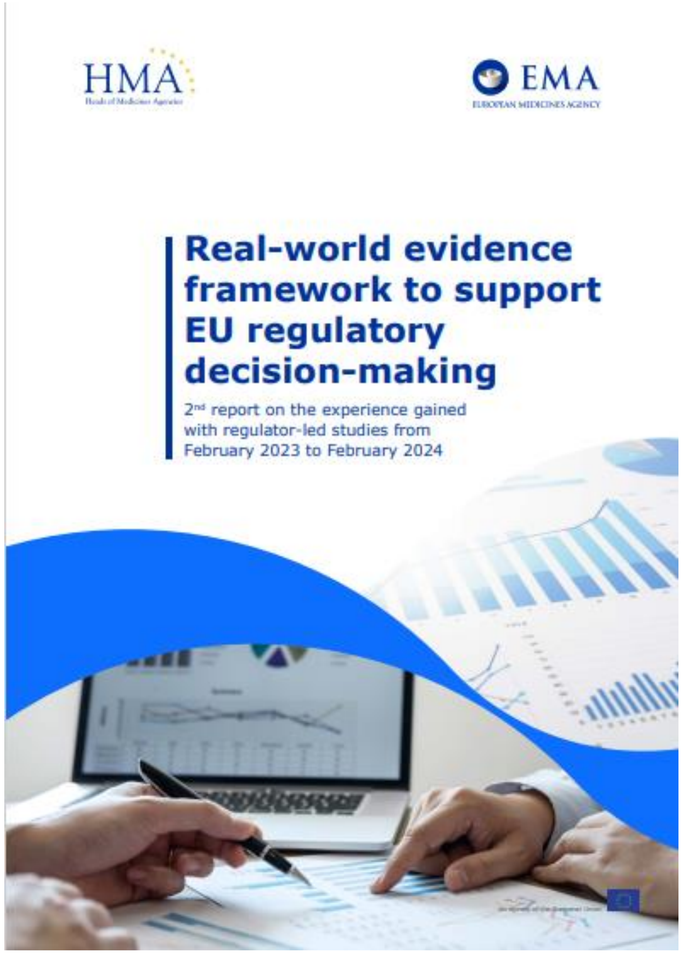
- Coordination Centre launched February 2022
- Onboarded **20 data partners** during the first 2 years
- **20+ studies** finalised
- Additional 10 data partners are foreseen to **be added each year** for 2024 and 2025

Reports on RWE experience



Published in June 2023

Period covered: **Sep 2021 to Feb 2023**



Published in July 2024

Period covered: **Feb 2023 to Feb 2024**

What did we do? Where are we?

Total number of RWD studies per pathway				
	Newly requested (Feb 2023-Feb 2024)	Completed (Feb 2023 – Feb 2024)		
DARWIN EU studies	38	9		
In-house studies	16	4		
Framework contract studies	6	9		
Total	**60**	**22**		

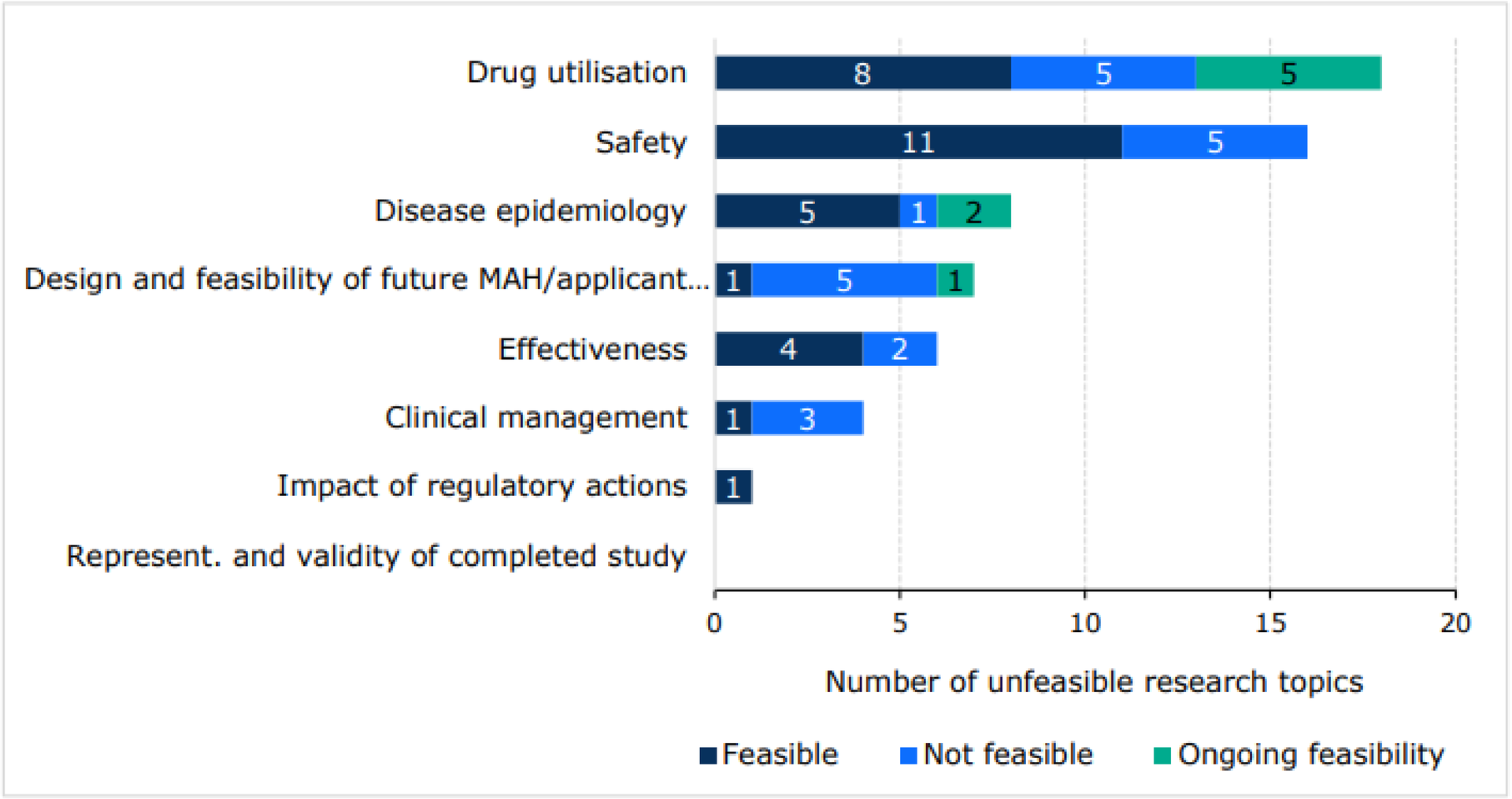
* From February to September 2024 (8-month period)

What did we do? Where are we?

Total number of RWD studies per pathway				
	Newly requested (Feb 2023-Feb 2024)	Completed (Feb 2023 – Feb 2024)	*Newly requested (since Feb 2024)**	*Completed (since Feb 2024)**
DARWIN EU studies	38	9	*42*	*12*
In-house studies	16	4	*6*	*4*
Framework contract studies	6	9	*5*	*3*
Total	**60**	**22**	***53***	***19***

* From February to September 2024 (8-month period)

Type of research topic by use case and feasibility status



HTA bodies / Payers –related studies

Multiple myeloma: Patient characterisation, treatments and survival in the period 2012-2022 [EUPAS105033]

OTS

Overall survival in patients with advanced or metastatic **non-small cell lung cancer** treated with selected **immuno-therapies as first line** [EUPAS1000000112]

Complex

New developments in 2024

- **Study** to investigate the risk of major CV events in obese/overweight patients treated with GLP1a compared to other therapies
  - Discussion after feasibility assessment to wait for more appropriate data
- **Technical meetings**
  - In place since May
  - Discussion on methodological part of complex studies to ensure the work performed is fit for decision making

European medicines agencies network strategy (EMANS) to 2028

Accessibility – to facilitate pathways for access to medicines through healthcare systems in the EU

Leveraging data, digitalisation and artificial intelligence – to improve decision-making, optimise processes and increase efficiency

Regulatory science, innovation and competitiveness – to create a regulatory and research environment that improves innovation and competitiveness of the EU's healthcare sector

Antimicrobial resistance and other health threats – to prepare the EU for potential threats including antimicrobial resistance

Availability and supply - to strengthen the availability of medicines to protect public and animal health

Sustainability of the network – to ensure that the network has available resources to support its scientific and regulatory decision making

The path to accessibility…

Accessible medicine authorised by regulators and then evaluated positively by other relevant authorities such as HTA bodies and payers

Regulators aims to facilitate the path to accessibility

- Evidence generated through development, authorisation and post-authorisation phases is relevant for HTA bodies and payers (Collaborating with HTA bodies and payers to generate such evidence)
- These bodies (as well as patients and healthcare professionals) aware of scientific considerations behind regulatory outcomes

New HTA regulation — To foster generation of robust evidence to serve different decision makers, continue collaborative work on methodologies, ensure communication about scientific considerations leading to regulatory outcomes

Goals for accessibility

Goals	How we will achieve them
Optimise the path to accessibility by working with other decision makers (HTA bodies and payers)	• Contribute to the successful implementation of the HTA Regulation • Foster the generation of robust scientific evidence to serve different decision makers (regulators, HTA bodies and payers) • Enhance communication with other decision makers about the scientific considerations leading to regulatory outcomes
Deepen engagement with healthcare policy makers on initiatives and research relevant to sustain health technology accessibility	• Contribute to initiatives exploring the perspective different stakeholders have about unmet medical needs and how they inform considerations about clinical significance, significant benefit and major contributions to patient care • Conduct research to better understand accessibility for medicines addressing unmet needs and how evidence requirements affect decision outcomes • Continue collaborative work on methodologies for the generation of evidence that is also relevant for health technology assessments

Thank you!

Further information

Contact me at patrice.verpillat@ema.europa.eu

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EUROPEAN MEDICINES AGENCY
SCIENCE MEDICINES HEALTH

09:50 CET

Plenary Session: The Policy Context for Real-World Evidence to Support Innovation in HTA

Moderated by François Meyer, RWE4Decisions Facilitator

Panel discussion: The Policy context for Real-World Evidence in the EU

Followed by Q&A

COUNCIL OF THE EU PERSPECTIVE

Enrique Terol García

Coordinating Advisor on Health, Permanent Representation of Spain to the European Union

REGULATOR PERSPECTIVE

Patrice Verpillat

Head of Real-World Evidence, European Medicines Agency (EMA)

PAYER PERSPECTIVE

Christoph Rupprecht

Head of Department for Health Policy and Health Economics, AOK Rheinland/Hamburg

PATIENT PERSPECTIVE

Anne-Pierre Pickaert

Member, Acute Leukemia Advocates Network

INDUSTRY PERSPECTIVE

Alexander Natz

Secretary-General, European Confederation of Pharmaceutical Entrepreneurs (EUCOPE)

11:25 CET

Plenary Session: RWE4Decisions work in support of the EU health agenda

Moderated by

Ashley Jaksa

VP Scientific Strategy and Partnerships, Aetion

11:25 CET

Plenary Session: RWE4Decisions work in support of the EU health agenda

Moderated by Ashley Jaksa, VP Scientific Strategy and Partnerships, Aetion

RWE4Decisions work in 2024

Karen Facey

RWE4Decisions Facilitator

11:25 CET

Plenary Session: RWE4Decisions work in support of the EU health agenda

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

Ashley Jaksa

VP Scientific Strategy and Partnerships, Aetion

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

Ashley Jaksa MPH

VP Scientific Strategy & Partnerships

Aetion, Inc.

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Scan the QR code with your phone to read the 2024 Stakeholder Actions to Generate Better RWE for HTA/Payers

New Stakeholder Actions to address what? Updated vision

Since 2020…

Decision-making environment, RWD infrastructure and RWE sciences have changed:

- More opportunities for secondary use of data
- Methodological advancements in building/reporting of RWE (highlighting potential to resolve decision-relevant uncertainties)

Stakeholders undergone work in the line of the original actions:

- improved the quality and accessibility of RWD
- analytical methods advances
- new guidance for researchers generating RWE
- EMA - major strides in data analytics and digital transformation

But has not always translated to efficient and robust use of RWE in HTA/payer decision-making.

Lack of harmonization between Regulators and HTA bodies/Payers and between HTA bodies and Payers remains a key challenge noted by stakeholders.

The HTA/Payer Community needs to work together to inform the health system of the specific RWD needs essential for their decision making and procurement process.

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Stakeholder Actions revision timeline


International Journal of Technology Assessment in Health Care

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

Karen M. Facey, Flia Ramanhelimo, Laura Batchelor, Marine Borchardt and Jo de Cock

Policy

Background

CAMBRIDGE UNIVERSITY PRESS

Publication of Actions for Stakeholders paper v1.0

2020

2023

Top10 most downloaded papers in IJTAHC in 2023

January 2024

Focus group discussions with each stakeholder group

HTA/Payers

Registries

RWD/Analytics

Clinicians

Industry

Patients

February 2024

Multi-stakeholder Roundtable on the focus group meetings outcomes

49 participants (incl representatives from 10 different HTA/Payer bodies)

March 2024

Webinar announcing public consultation on the revised actions

133 attendees

April - June 2024

Public consultation feedback period

11 responses received

July-October 2024

Writing process and co-authors' alignment, with at least 1 representative from each stakeholder group

Revision of RWE4Decisions Vision and Mission with the Steering Group

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Stakeholder Actions – Dissemination

October 2024

Submission of revised Stakeholder Actions paper in IJTAHC

International Journal of Technology Assessment in Health Care

14 November 2024

Launch revised Stakeholder Actions at the RWE4Decisions Annual Symposium

17 November 2024

Discuss revised Stakeholder Actions at the ISPOR RWE Summit

ISPOR
Improving healthcare decisions

Early 2025

Publication of the new Stakeholder Actions paper

2025 and beyond

Dissemination and Implementation by Stakeholder Groups

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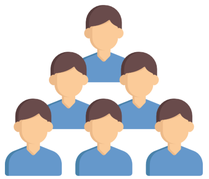
Stakeholder groups

Working within the principles of collaboration and transparency, each stakeholder can undertake actions to support the use of real-world evidence in Payer/HTA decisions about highly innovative technologies:



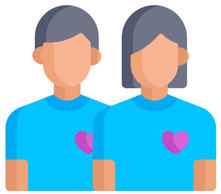
Payers/HTA Collaborations

National Payer/HTA bodies

Pharmaceutical Industry

Registry-holders

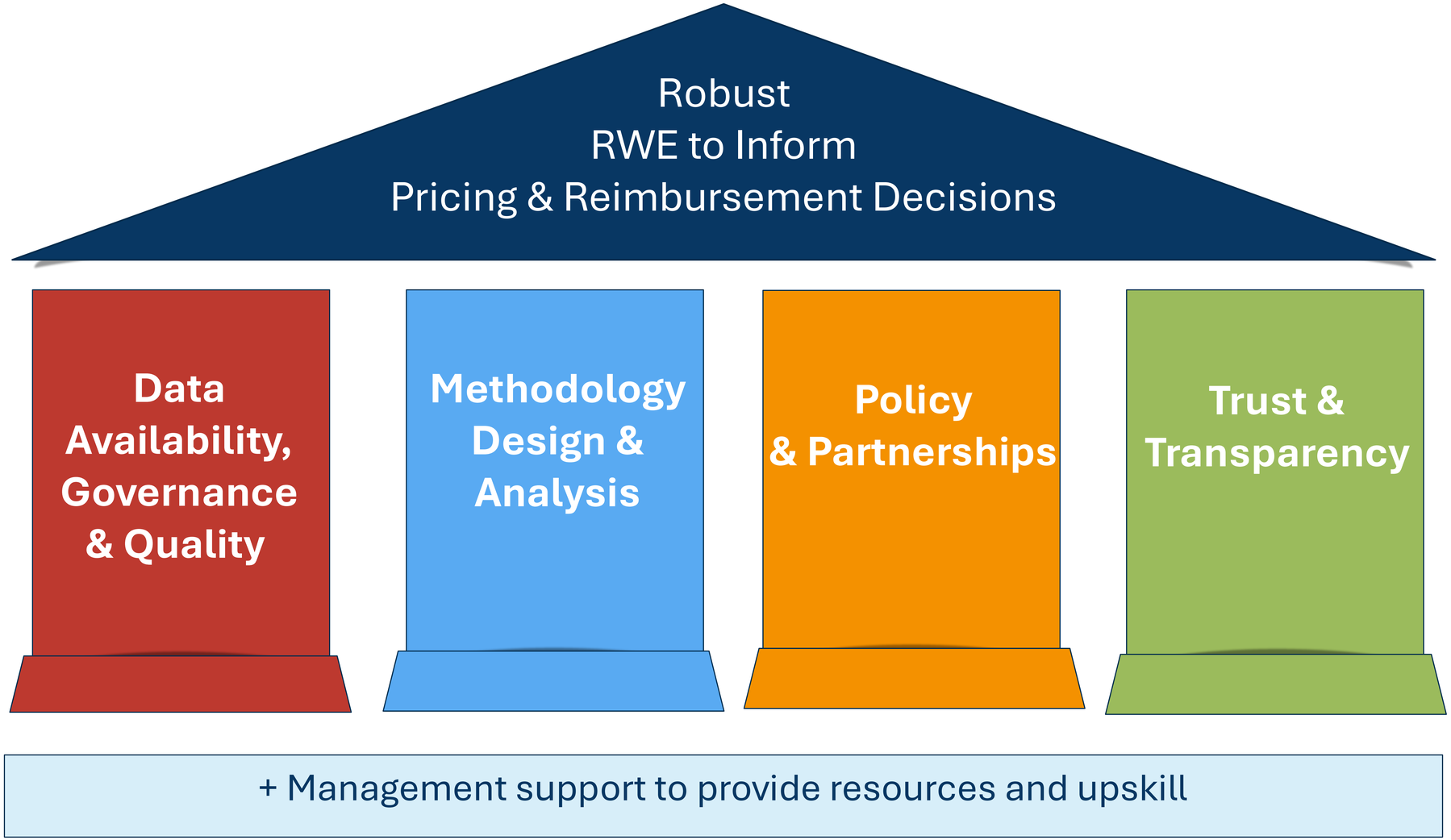
Clinical teams



Patient groups

RWD / analytics groups

Four pillars to support development of robust RWE for HTA/Payer decision-making (2021)



Capkun, G., Corry, S., Dowling, O., Asad Zadeh Vosta Kolaei, F., Takyar, S., Furtado, C., . . . Facey, K. (2022). Can we use existing guidance to support the development of robust real-world evidence for health technology assessment/payer decision-making? *IJTACC, 38*, E79. doi:10.1017/S0266462322000605

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11:25 CET

Plenary Session: RWE4Decisions work in support of the EU health agenda

Moderated by Ashley Jaksa, VP Scientific Strategy and Partnerships, Aetion

Launching the new RWE4Decisions Stakeholder Actions to Generate Better Real-World Evidence for HTA/Payer Decisions

Followed by Q&A

NATIONAL HTA PERSPECTIVE

Piia Rannanheimo

Chief Specialist, Finnish Medicines Agency (FIMEA)

COLLABORATIVE HTA PERSPECTIVE

Shaun Rowark

Associate Director for Data Access and Analysis, National Institute for Health and Care Excellence (NICE)

PATIENT PERSPECTIVE

Stefan Joris

Board Chairman, RaDiOrg – Rare Disease Belgium

INDUSTRY PERSPECTIVE

Andre Vidal Pinheiro

Vice-President and Head of Patient Value & Access – Europe & Canada, Takeda

Key Actions - National Payers/HTA Bodies

Policy & Partnerships

1.2 Overcome fragmentation and lack of collaboration between HTA bodies and payers by implementing the necessary infrastructures, aligning processes, and upskilling competencies for effectively requesting, producing, and utilizing real-world evidence.

Data Availability, Governance & Quality

1.5 Influence national developments on the secondary use of health data. Communicate HTA/Payer needs regarding, for example, types of data, data linkage and data quality, ensuring these needs are considered and integrated into national governance frameworks.

1.7 Publish examples where RWE has influenced pricing and reimbursement decisions or reassessments. Also share case studies that identify methodological areas requiring development.

Key Actions - Payers/HTA Bodies Collaboratives

Policy & Partnerships

2.4 Work with Regulators to understand and influence their international activities to develop harmonized methods and guidance for RWD collection. In Europe, incorporate RWD/E needs and guidance in the implementation of the HTA Regulation through Joint Scientific Consultations and Joint Clinical Assessments.

2.7 Collaborate with companies, clinical teams, academia and other stakeholders on study protocols, study governance, analyses, and reporting to encourage a common understanding of HTA requirements and to promote open access to documents and findings.

Data Availability, Governance & Quality

2.8 Collaborate with regulators on common frameworks for data quality assessment, data standardisation efforts and methodologies for feasibility assessment. Advise health data holders of the common requirements so that they can develop their datasets accordingly.

Key Actions – Patient Groups

Policy & Partnerships

5.4 Support development of a process for iterative multi-stakeholder dialogues throughout the lifecycle of a medicine to encourage alignment of views on identification, collection, analysis and evaluation of RWD for decision-making.

Data Availability, Governance & Quality

5.6 Disseminate clear, unbiased, patient-relevant information about RWD and RWE to patient communities, including the value of secondary use of data and information to support Post-Launch Evidence Generation.

Policy & Partnerships

5.1 Ensure that opportunities and resources to develop patient expertise in the field of RWD are clearly communicated to the patient community to develop skills that support multi-stakeholder and patient-centred generation of real-world evidence (RWE) to inform health technology assessment (HTA) and to engage in policy and system developments relating to use of health data.

Key Actions – Pharmaceutical Industry

Trust & Transparency

3.2 Ensure transparency around the design, conduct, and analysis of RWE studies that are agreed to be pivotal to health technology assessment (HTA)/Payer decision making, e.g. using published tools to document data capture, management and analysis, following RWE guidance/frameworks.

3.5 Continue to drive discussions about use of, and alignment of, Outcomes-Based Managed Entry Agreements (OBMEA)/Post-Launch Evidence Generation (PLEG).

Data Availability, Governance & Quality

3.6 Explore use and analysis of digital apps to capture patient-relevant outcomes, particularly to inform OBMEA.

Methodology Design & Analysis

3.9 Engage and support operationalisation of the HTA Regulation to highlight need for RWE in the first two tranches of JCAs and encourage development of clear guidance about assessment of RWE in the EU HTA context.

12:35 CET

Closing Session: Way forward – RWE4Decisions agenda to deliver in future

Next steps for the implementation of EU HTA Regulation

Followed by Q&A

Carlos Martín Saborido

Director of the HTA Agency, Agencia de Evaluación de Tecnologias Sanitarias (AETS)

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secretariat@rwe4decisions.com

Next steps for the implementation of EU HTA Regulation

Carlos Martín Saborido

HTA Agency ISCIII

Madrid, Spain

What should be the next steps for the implementation?

TWO IDEAS

- Use of relevant information to be included along with the JCA: RWE
- Work in parallel to the development of the JCA

THREE ACTIONS

Are efficacy/safety data enough for decisions on Rare Diseases?

Value = Efficacy + Safety + Use of reosurces + Care load +…

Specific data collection for reimbursment decisions:

- HRQoL
- Use of resources to cope with the condition

When do we start talking/negotiating?

Currently: Late

Early dialogue to address

- Uncertainty coverage
- Anticipation about financial agreement
- Discussion/agreement about effects modifiers (MAIC, STC)

What is leading the early dialogue?

Horizon Scanning

For rare diseases:

- As soon as something is identified
- Early collection of data (incidence, prevalence, use of resources…)

That allows:

- Data about the correct comparison
- Epidemiologic data (BIA)

let's talk

12:35 CET

Closing Session: Way forward – RWE4Decisions agenda to deliver in future

Conclusions and Payer perspectives

Jo de Cock

Senior Adviser and former CEO, Belgian Institute for Health and Disability Insurance (INAMI-RIZIV)

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RWE4Decisions Symposium

Thank you for your contributions!

Lunch will follow

The recording will be available on our website
www.rwe4decisions.com

Stay in touch on
secretariat@rwe4decisions.com
and keep up to date on our Linkedin, @RWE4Decisions