



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

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Overcoming Obstacles: Charting the Path Ahead

ISPOR EU Real-World Evidence Summit 2024

17 November 2024

RWE4Decisions REAL WORLD EVIDENCE

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International Journal of Technology Assessment in Health Care

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Policy

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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 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.

REAL WORLD EVIDENCE
RWE4Decisions



New Stakeholder Actions to address what? Updated vision

Since 2020...

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

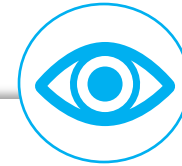
- More opportunities for secondary use of data
- Methodological advancements in building/reporting of RWE (highlighting potential to resolve decision-relevant uncertainties)

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.

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.

Over 2024, RWE4Decisions and its Learning Network co-developed an updated set of Actions for Stakeholders.



HTA/Payers



Clinical
Teams



Registries



Industry



Patients



RWD/
Analytics

Revised RWE4Decisions Actions for Stakeholders to generate better Real-World Evidence for HTA/Payers



Publication of Actions for Stakeholders paper v1.0

2020

2023

Top10 most downloaded papers in IJTAHC in 2023

Focus group discussions with each stakeholder group

- HTA/Payers
- Registries
- RWD/Analytics
- Clinicians
- Industry
- Patients

January 2024

February 2024

Multi-stakeholder Roundtable on the focus group meetings outcomes
49 participants (including representatives from 10 different national HTA /Payers)



Webinar announcing public consultation on the revised actions

133 attendees

March 2024

April - June 2024

Public consultation feedback period
11 responses received, from National HTA/Payer bodies, Clinicians, Patient Groups, Academia, Industry.



Re-issue Actions for Stakeholders paper v2.0 at RWE4Decisions Symposium

November 2024

2025

Publication of new paper and Dissemination at EU and national levels

Revision of RWE4Decisions Vision and Mission with the Steering Group



The New Actions for Stakeholders to generate better Real-World Evidence for HTA/Payers

Scan the QR code to read the Actions!





The 2024 actions: new driving considerations

Promote a joint vision of RWE (what can be produced, by who and for what) and more harmonisation

Actions are still destined to specific groups of stakeholders, but based on a collaborative approach:

- HTA bodies/payers
- The Pharmaceutical industry
- Clinical Teams
- Patients
- Registry holders
- Analytics experts

Actions are highly intertwined and will require multi-stakeholder collaboration

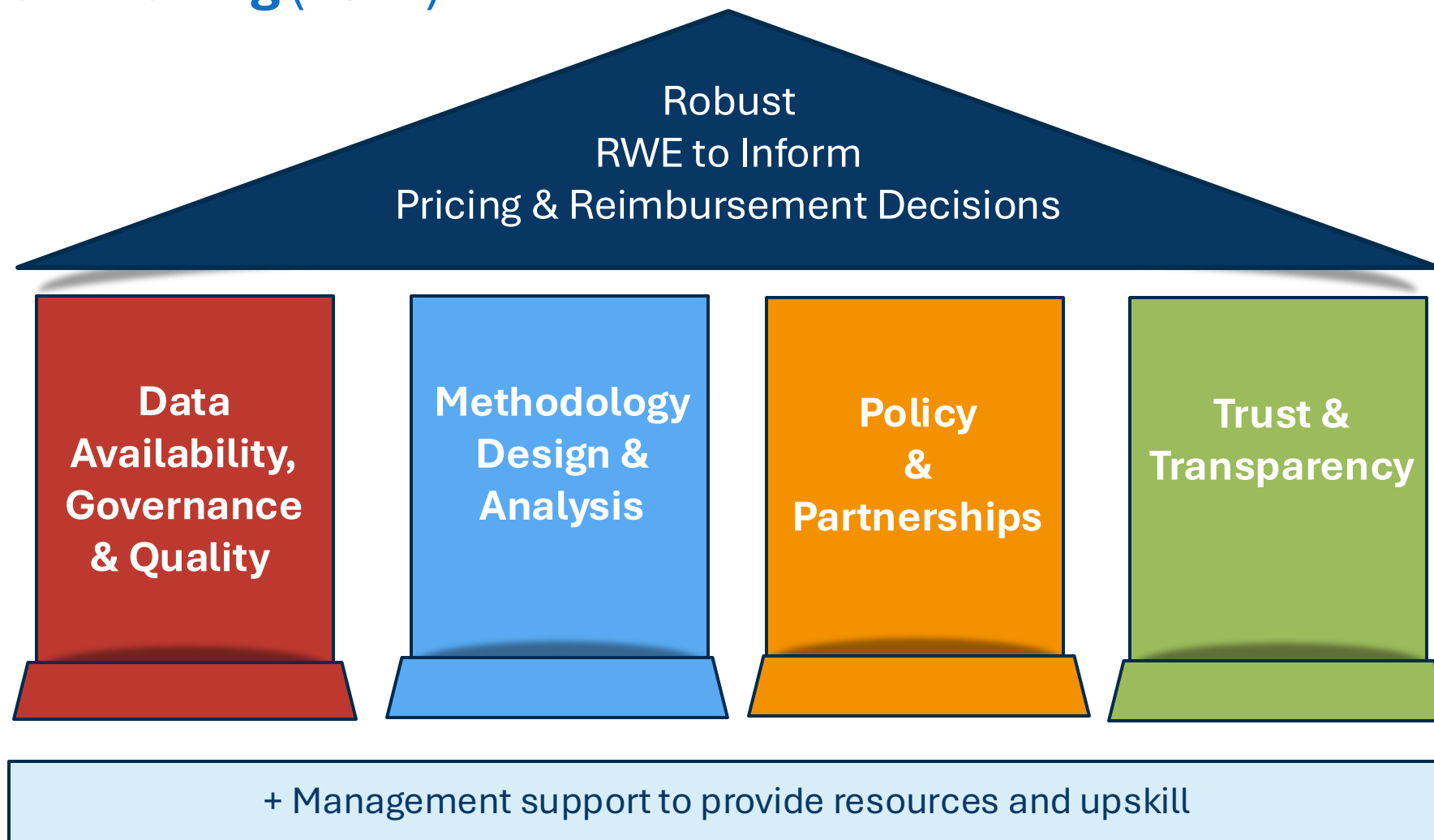
For many actions, multiple stakeholder groups should be involved and share responsibilities; the onus should not just fall on HTA bodies and/or industry.

More tailored to the specific and current challenges of each stakeholder group

Co-authored by Ashley Jaksa (Aetion), Pii Rannanheimo (Fimea), Laurie Lambert (Canadian Drug Agency), Matti Aapro (Genolier Cancer Center), Niklas Hedberg (TLV), Victoria Hodgkinson (Canadian Neuromuscular Disease Registry), Matias Olsen (EUCOPE), Karen Facey, François Meyer, Alina Pavel (RWE4Decisions)



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



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 - collaborative Payers/HTA bodies

Policy & Partnerships

2.2 Develop a joint voice on RWD issues to feed into policy and system developments such as the European Health Data Space (EHDS) and the Data Analysis and Real-World Interrogation Network (DARWIN-EU) and the implementation of the HTA Regulation, with clear accountability for this task potentially assigned to an entity such as NCAPR.

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.

Methodology Design & Analysis

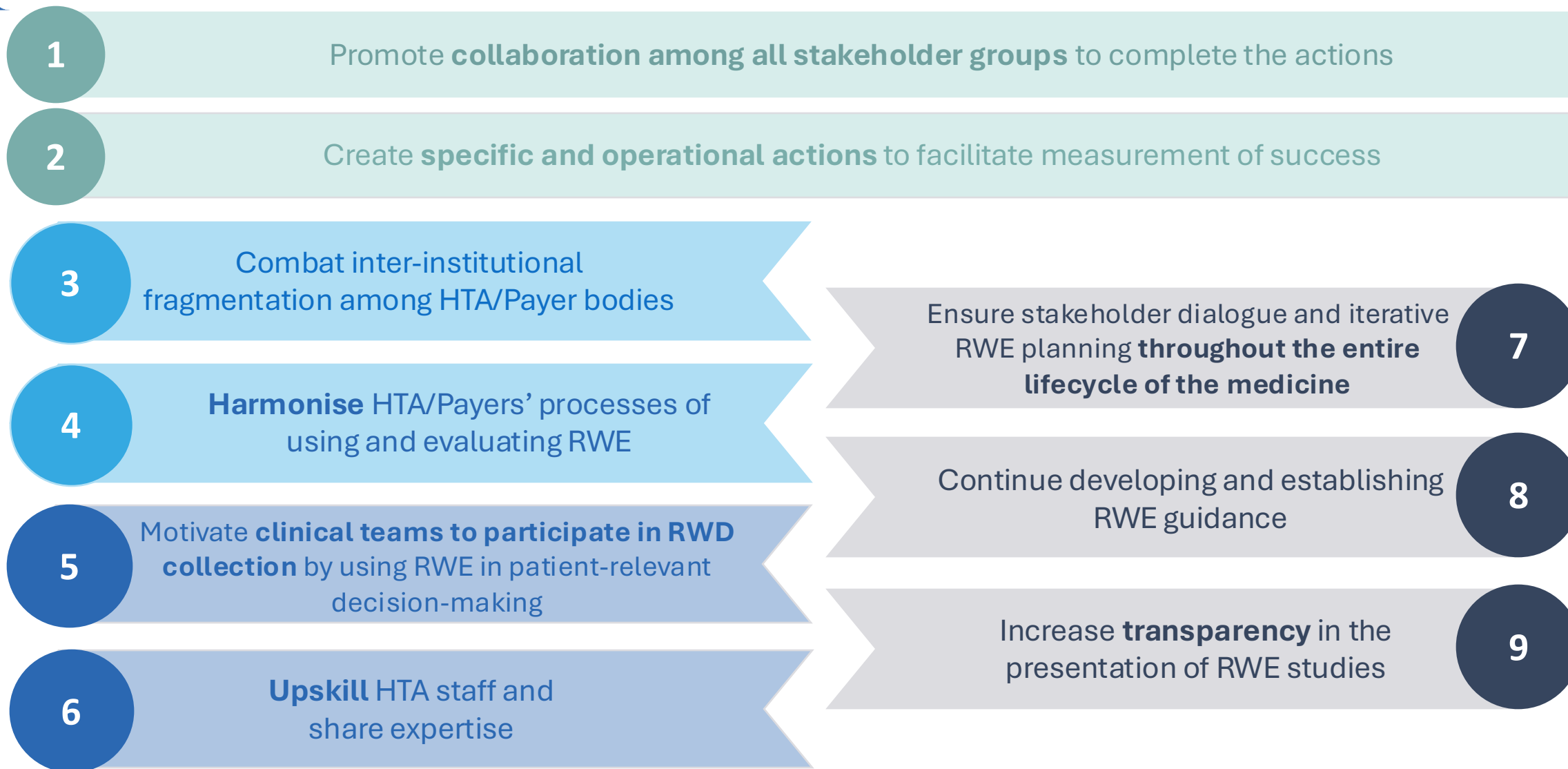
2.9 Pilot the use of existing RWD/E methods guidance and tools in assessments. Share feedback with guidance authors and HTA/Payer community.

Trust & Transparency

2.11 Compile available documents (protocols and reports) describing examples of post launch RWD collection and RWE generation required by HTA/payers, utilizing an existing portal if available.



Key learnings and trends identified





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