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

RWE4Decisions Multi-stakeholder Learning Network, November 2024

1. Payers and HTA Bodies: National Payers/HTA bodies

POLICY and PARTNERSHIPS

1.1 Create a shared vision that conceptualizes a future state for the use of real-world data (RWD) in health technology assessment (HTA)/Payer processes. Ensure that clear roles and responsibilities are designated for oversight and achievement of the shared vision.

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 and GOVERNANCE

1.3 Collaborate with other HTA bodies and Payers (e.g. via networks such as the National Competent Authorities for Pricing and Reimbursement (NCAPR) or the Medicine Evaluation Committee (MEDEV)) and regulatory authorities to align post-launch evidence generation (PLEG) requirements needing national data collection. Focus on critical endpoints and requirements for data quality.

1.4 Initiate and engage in multistakeholder discussions with companies about RWE generation plans. Determine which data are transferable and identify additional local RWD that may be needed to inform decision-making, aligning with other jurisdictions when possible.

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.

1.6 Require that feasibility assessments, study protocols, details of data extractions and study reports are made publicly available.

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

2. Payers and HTA Bodies: Payer/HTA collaborations

POLICY and PARTNERSHIPS

2.1 Use HTA/Payer collaboratives (such as Joint Nordic HTA-Bodies, the Beneluxa initiatve etc) to encourage and enhance joint work on use of RWD in initial access decisions, managed entry agreements and re-assessments.

2.2 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.

2.3 Co-create joint learning platforms with other decision-makers and stakeholders in the RWD/E community.

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.

2.5 Collaborate to expand Early Dialogue/scientific advice/Joint Scientific Consultation to cover RWE generation throughout the product life cycle to address uncertainties in clinical and cost effectiveness. Recognise the need for agile processes to adapt to evolving knowledge, treatment options and RWD and RWE requirements.

2.6 Encourage and support industry to consider possibilities to expand post-authorisation studies and data collection plans intended to support regulatory decision making to also address HTA/Payer evidence needs.

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 and GOVERNANCE

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.

METHODOLOGY

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

2.10 After piloting existing guidance, develop harmonized RWE guidance jointly with academia and industry, particularly to support implementation of the HTA Regulation. Review the guidance regularly to take account of new methodological developments and experience of use (living guidance).

TRUST and 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.

3. Pharmaceutical Industry

TRUST and TRANSPARENCY

3.1 Discuss plans for RWE generation as part of industry (integrated) evidence plans at scientific advice meetings. Do this at several points during the lifecycle of the medicine, to develop understanding of what RWE may be valuable to HTA/Payers as clinical evidence and knowledge about the technology/disease evolves.

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.

POLICY and PARTNERSHIPS

3.3 Work together across company functions and national affiliates involved in RWE generation and analysis to develop a common understanding of national/regional HTA/Payers needs for RWE.

3.4 Lead discussions about transportability of real-world data (RWD) across borders and support efforts to align data collection requirements across jurisdictions.

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 and GOVERNANCE

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

3.7 Collaborate across companies (e.g. in a specific disease area or for a class of medicines) to agree on synergies in methods and processes for RWE in that specific case (e.g. core datasets, analytical approaches, etc).

3.8 At an early stage of product development, engage with clinical networks to create or further develop registries and databases following unified data standards, to collect high quality and interoperable data.

METHODOLOGY

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.

3.10 Share, publish and enable discussion of case studies to show how RWE has been assessed and used in HTA/Payer decision-making (in assessment and post-launch), focussing on specific issues (e.g. external control arms, transportability), and challenging cases scenarios (e.g. rare disease) etc.

4. Clinical Teams

POLICY and PARTNERSHIPS

4.1 EU clinical networks should engage in developments related to the European Health Data Space to support public and political awareness of the value of secondary use of health data.

4.2 EU clinical networks and clinical trials collaborative groups (such as European Reference Networks, the EU cancer mission networks, the European Organisation for Research and Treatment of Cancer and other disease specific study groups) should systematically involve patients to collect patient relevant outcomes including nutritional status and co-morbidities, and collaborate with regulators and Payer/health technology assessment (HTA) bodies to ensure data collection systems are fit for all purposes, including HTA requirements pre and post launch.

4.3 Medical faculties should include educational programmes on the value of health system data to improve delivery of care, patient outcomes and inform health system decision making, such as HTA evaluations of new healthcare interventions.

DATA AVAILABILITY and GOVERNANCE

4.4 Clinical networks and study groups should advise on the most suitable and efficient way for health systems to collect real-world data (RWD) to avoid multiplicity of data entry and clarify the support clinical teams require to collect good quality RWD.

4.5 Clinical networks and clinical trials collaborative groups should encourage health systems to involve clinical teams and patients in the design of data collection systems and associated governance structures to ensure processes are efficient and clarify the support clinical teams require to collect good quality real world data.

4.6 Clinicians should seek to ensure that local information governance systems are designed to deliver optimal care for patients (across providers and departments), and to encourage informed consent processes based on unified ethical principles that are intelligible to patients, in all required languages.

5. Patient Groups

POLICY and 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.

5.2 International patient groups should continue to engage in real-world data (RWD) initiatives, such as Innovative Health Initiatve (IHI) projects, regulatory and HTA led activities, and policy developments such as the European Health Data Space (EHDS).

5.3 Seek to influence the implementation of the EHDS and understand its implications for national data collection systems, in particular to ensure that patients have access to their own data.

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 and GOVERNANCE

5.5 Support the development of efficient informed consent processes for secondary use of health data. This will necessitate a rethinking of the type of consent model in use from 'broad to 'dynamic' to encourage accelerated and increased patient participation.

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.

METHODOLOGY

5.7 Engage with clinicians, academics and decision-makers to discuss how patient-relevant data from novel collection methods (such as wearables, apps etc) can be used in decision-making, to help build a predictable pathway for use of these novel RWD collection approaches.

5.8 Patient Groups who might also be disease registry holders or have developed disease specific data collection approaches, should agree with stakeholders how to integrate such evidence with other data for highly innovative medicines.

6. Disease Registry Holders

POLICY and PARTNERSHIPS

6.1 Participate in multi-stakeholder dialogues about real-world evidence (RWE) generation for a specific medicine, or group of medicines, to discuss the potential for a disease registry to be used for regulatory and health technology assessment (HTA)/Payer purposes and ensure realism about data quality (availability for main outcomes and trade-offs for different data capture algorithms).

6.2 Form multi-stakeholder partnerships to support use of data for HTA/payer purposes, including public/private partnerships.

TRUST and TRANSPARENCY

6.3 Engage with HTA/Payers and industry to explain the construct and purposes of disease registries, discuss their potential and limitations, and agree with HTA/Payers how disease registries and RWE studies will be assessed, building on existing tools (e.g. tools to report registry quality overall, and fitness-for-purpose evaluations for individual RWE studies).

6.4 Share and publish case studies of where real-world data (RWD) from disease registries has been used in to support HTA/Payer decision making.

DATA AVAILABILITY and GOVERNANCE

6.5 Develop governance processes that enable sharing of disease registry data and linkage to other data sources (e.g. administrative data) for HTA/Payer purposes (including patient consent mechanisms, protocols, data management etc).

6.6 Align to RWD standards to ensure data meets quality standards required by HTA bodies.

7. RWD/Analytics Groups

METHODOLOGY

7.1 Support development of a repository of empirical evidence/case law about what real-world evidence (RWE) was fit for purpose in health technology assessment (HTA) and what was not, as well as RWE case studies that highlight data quality and methodology examples.

7.2 Continue to operationalise and share/publish methodologies that address known HTA/Payers' concerns with use of RWE through HTA-friendly tools, demonstration projects, case studies etc.

7.3 Build RWE analytics knowledge base and support RWE assessment and generation within HTA bodies linking to new policy initiatives such as the HTA Regulation.

7.4 Work with HTA bodies to share and operationalize the implications from available demonstration projects (e.g., emulation studies, newly developed methods, tools, repositories etc) to build mutual understanding and trust in RWE; explore which are most helpful or need adaptation and support collaboration to create a harmonized RWE toolkit.

7.5 Raise awareness of differences between HTA body RWE frameworks and how these differences relate to differences in HTA body remits and RWE needs. Where appropriate, engage in dialogue with HTA bodies to encourage harmonization of fit-for-purpose methods.

7.6 Where there is substantial evidence and agreement across stakeholders on use cases, standards, and analytical methods and where articulating guidance would benefit researchers, support HTA bodies in developing detailed published guidance.

7.7 Create a standard world-wide library of definitions for key aspects covering diagnoses, outcomes, covariates etc and algorithms that have been validated.

TRUST and TRANSPARENCY

7.8 Work with industry to ensure they are following published standards/best practices for RWE generation (e.g., transparency, data quality, etc).

7.9 Work with data custodians to explain the requirements of decision-makers to clearly demonstrate data quality and encourage standardised documentation they can use for all RWE studies.

RWE4Decisions is a payer-led multi-stakeholder learning network, which has developed **stakeholder actions** that will better enable the use of real-world evidence in HTA/payer decisions about highly innovative technologies. The work has been commissioned by the Belgian National Institute of Health and Disability Insurance (NIHDI) and is led by a multi-stakeholder Steering Group with a wider community of contributors including HTA bodies and payers, regulatory agencies, patient groups, clinicians, industry, analytics experts and academic experts/researchers.

The RWE4Decisions Secretariat is provided by FIPRA, with sponsorship by the European Confederation of Pharmaceutical Entrepreneurs (EUCOPE), AstraZeneca, Boehringer Ingelheim, Gilead, MSD, Novartis, Pfizer, Roche and Takeda.

For further information and to read an online version of the Stakeholder Actions, visit our website, www.rwe4decisions.com.

What are you are doing to progress learnings on the use of RWE?

Contact us at **secretariat@rwe4decisions.com** to join the RWE4Decisions Learning Network.

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