REAL WORLD EVIDENCE
RWE4Decisions Symposium

Real-World Evidence over the Medicine's Lifecycle to Inform HTA / Payer Decisions

Event Report

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Executive Summary

On 23 November 23, the RWE4Decisions Learning Network held its Annual Symposium entitled "Real-World Evidence over the Medicine's Lifecycle to inform HTA/Payer decisions" in a hybrid format. This event brought together a diverse in-person audience, including representatives from HTA bodies, Payers, European and national policymakers, international organisations, healthcare technology developers, clinicians, researchers, and patient advocates.

Throughout the symposium, the focus remained firmly on the potential value of RWE, as complementary to traditional clinical trials, to inform decisions about highly innovative medicines; addressing evidence gaps, ensuring optimal treatment, and improving the efficiency of healthcare systems, particularly for rare diseases and Advanced Therapeutic Medicinal Products.

To inform decision-making RWE needs to be generated from well-planned studies that answer specific research questions aimed at resolving decision-relevant uncertainties. Ideally these studies are designed well in advance with all stakeholders to agree a minimum RWD dataset, so that collection of high quality RWD from trusted sources can be organised and incentivised. This requires aligning, anticipating, advancing, and adapting methodologies for RWE.

There was enthusiasm from all stakeholders to see the development of the European Health Data Space (EHDS), noting its transformative potential in the EU. However, challenges pertaining to data governance and interoperability were acknowledged, calling for cohesive policies and frameworks to maximise the EHDS's impact.

Distinguished initiatives like DARWIN-EU were showcased, illustrating how RWE can be effectively leveraged to answer regulatory and HTA/Payer questions, such as disease prevalence, existing treatments etc. The symposium also shed light on the potential for well organised Early Access programs to be a source of local RWD for HTA/Payers.

A recurring theme throughout the symposium was the criticality of collaboration among stakeholders to develop a unified understanding among regulators, patients, HTA bodies, Payers, and industry regarding the role and potential of RWE to inform a Payer decision. This requires more dialogues among stakeholders to discuss challenges in developing RWE, such as lack of interoperability, reasons for poor data quality and impacts of different methodological approaches, and agree optimal solutions. This should include learning from HTA case studies where RWE has influenced decision-making, and where it has been insufficient. This could support development of harmonised guidance on generation and assessment of RWE, particularly in the context of the HTA Regulation.

Payers could play a crucial role among stakeholders to explain the potential value of high quality RWD not only to inform pricing and reimbursement decisions about highly innovative medicines, but to optimise treatment and care and thus support health system sustainability. This is a dynamic and responsive continuum of evidence generation over the life cycle of a highly innovative medicines. It requires a reshaping of the practice of medicine to include meticulous data entry as an integral part of healthcare provision, emphasising the societal value of accurate data collection and encouraging learning within the system. This requires investment in capacity development and infrastructure to support collection of RWD in all sectors.

INTRODUCTION FROM CO-MODERATORS

HANS-GEORG EICHLER

Consulting physician, Association of Austrian Social Insurance Institutions

KAREN FACEY

Senior Adviser HTA, FIPRA
RWE4Decisions Facilitator

The RWE4Decisions Annual Symposium commenced with opening remarks from co-moderators Dr. Hans-Georg Eichler and Dr. Karen Facey, members of the RWE4Decisions Steering Group. They set the tone for an insightful exploration into the realm of Real-world evidence (RWE) over the Medicine's Lifecycle to inform HTA/Payer decisions. They highlighted the aim of the RWE4Decisions learning network to bridge gaps in clinical development of highly innovative medicines, resolve uncertainties through post-launch evidence generation, stressing the need for stakeholders to collaborate and encourage transparency in processes to build robust RWE that can inform optimal care and support health system sustainability.

DATA POLICIES AND PATIENT ACCESS - SPANISH EU PRESIDENCY PRIORITIES

ENRIQUE TEROL GARCÍA

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

Dr. Enrique Terol García outlined the public health priorities of the Spanish Presidency of the Council of the European Union (EU). To strengthen the capacities of health systems in the EU, the Presidency focused, amongst others, on digital health. A critical component of this, is the European Health Data Space Regulation (EHDS). The aim is to reach a consensus between all EU Member States, giving the European Council a mandate to start negotiations with the EU Parliament within the current political term.

Dr. Terol emphasised that the EHDS is an ambitious piece of legislation, in its approach, and dimension, globally. It aims to address the use of health data in three key environments: patient rights and protection, healthcare systems' organisation and provision of care, as well as the IT infrastructural dimension. The pivotal role of the EHDS in unleashing the potential of health data in Europe will foster better collaboration, innovation, and a data-centric healthcare system across the EU. It will ensure enhanced and secure care by granting citizens access and control over their health data and facilitating cross-border data exchange for improved healthcare coordination. He elaborated on primary uses of data (citizens' access to their health data for personalised health care) and secondary uses (anonymised or pseudonymised data benefiting diverse stakeholders for research, innovation and policymaking), while acknowledging the complex challenges around the possible need to find agreement on an opt-in/opt-out

consent model for data sharing, transition periods for data exchange, harmonisation of interoperability criteria, and the substantial implementation costs.

THE POLICY CONTEXT FOR REAL-WORLD EVIDENCE

MARCO MARSELLA

Director of Digital, EU4Health and Health Systems Modernisation, DG SANTE, European Commission

PATRICE VERPILLAT

Head of Real-World Evidence Workstream, European Medicines Agency

DIMITRIOS ATHANASIOU

Board Member, World Duchenne Organization

ALEXANDER NATZ

Secretary General, European Confederation of Pharmaceutical Entrepreneurs (EUCOPE)

The role of Real-World Evidence for Sustainable Healthcare Systems

Mr. Marco Marsella emphasised the immense significance of the EHDS and its potential benefits for the community of HTA bodies and Payers. He underlined how the COVID-19 pandemic highlighted the crucial role of health data and how the EHDS aims to open up and share healthcare data for research, innovation, and policymaking in a trustworthy environment. Three components will be key for successful implementation of the EHDS: the legal framework, the procedures and mechanisms necessary for operationalisation, and the enabling infrastructure.

Mr. Marsella emphasised the necessity for a level playing field regarding definitions of Real-World Data (RWD) and RWE, and ongoing work to integrate these into EU legislation. The existing impact of RWE in the pharmaceutical and medical device spaces was highlighted, citing examples where RWD significantly influences clinical evaluations and insights generation. Mr. Marsella highlighted policy and research initiatives complementing RWE use, underscoring ongoing efforts to understand methodological frameworks and funding research for this purpose. He pointed out the importance of accompanying regulations with advancements in methodological application of RWE. He concluded by expressing the Commission's eagerness to collaborate with the HTA/Payer community, seeking insights and assistance from RWE4Decisions to navigate the evolving landscape of the EU HTA Regulation.

Scaling-up Real-World Evidence generation in Europe – Data Analysis and Real-World Interrogation Network (DARWIN EU)

Dr. Patrice Verpillat provided an update on DARWIN EU, focusing on the interaction between the initiative and HTA bodies and Payers. He began by sharing the 2025 vision for RWE utilisation, emphasising the need to enabling its use and establish its value for regulatory purposes, with EHDS as a critical component. DARWIN EU, initiated in 2022, aims to expand access to data sources and generate RWE for various use cases. Dr. Verpillat detailed three pathways for generating RWE: EMA in-house studies using primary care data, studies through EMA framework contracts with research organisations, and DARWIN EU collaborations with multiple data partners. He underscored how RWE supports decision-making by

elucidating the clinical context, planning and validating studies, and investigating associations and impacts of regulatory actions. Dr. Verpillat outlined DARWIN EU's role in supporting HTA/Payer decision-making, reflecting engagement with the community at a workshop in October 2022 that addressed concerns about RWD quality, the growing volume and variety of RWD, and the transparency of DARWIN-EU's operations. To conclude, Dr. Verpillat presented two pilot studies based on HTA/Payer questions re lung cancer treatment effectiveness and the natural history of multiple myeloma, showcasing DARWIN-EU's practical applications for HTA/Payer decision-making. He noted that EMA's newly published Data Quality Framework is an important resource for all stakeholders and will be followed by sub report on RWD quality in 2024.

Patients' perspectives

Mr. Dimitrios Athanasiou highlighted that many patient organisations recognise the value of real-world data to document various aspects of patient care and improve and align care pathways, particularly in rare diseases. The World Duchenne Organisation (WDO) has made substantial investment into collection of real-world data collection and been able to obtain a wealth of data that has helped identify effective treatments. However, decision makers often don't have clear processes for engaging with patient groups to obtain this form of evidence, their processes are geared to commercial sponsors. This is a challenge. This limitation is especially frustrating considering the breadth of developments in rare disease research that is led by patient organisations, ranging from animal models to clinical trials and digital outcomes, yet being unable to integrate their data into regulatory processes. His narrative vividly articulated the struggles and need for trust-building with patient communities to encourage active data contributions, but the value of the real-world data to identify good patterns of care and develop them further. Mr. Athanasiou's presentation underscored the indispensable role of patient engagement, highlighting that without active buy-in, the data pool remains underutilised. His call for expert guidance on effectively engaging patient organisations in data sharing resonated strongly, emphasising the necessity for a trust framework to facilitate successful collaboration and data integration. Mr. Athanasiou's impassioned plea for patient involvement in the integration of their data stressed the need for a more inclusive and participatory approach to shaping healthcare decisions.

Health technology developers' perspectives

Dr. Alexander Natz highlighted the swift progression of RWE within the healthcare industry over the past decade, emphasising its increasing significance. RWE has become an integral part of various policy initiatives, including the EHDS, the General Pharmaceutical Legislation, and the HTA Regulation. From an industry standpoint, Dr. Natz stressed the importance of incorporating multi-stakeholder views in defining a robust dataset that caters to the needs of patients, regulators, HTA bodies, payers, physicians, and the industry while also acknowledging the complexity in achieving this ideal scenario. He provided three examples where RWE has been pivotal. Firstly, during the pandemic, RWE significantly contributed to vaccine development and decision-making providing information about vaccination rates, effectiveness, and the suitability of vaccines for different patient populations. Secondly, in rare diseases, leveraging RWE from broader databases at the time of marketing can support assessment of treatments' long-term effects. Lastly, in value-based pricing, especially in chronic treatments and gene therapies like haemophilia, a robust dataset from RWE enables monitoring long-term effects and outcomes, crucial for payers' decisions. Dr. Natz highlighted the importance of multi-stakeholder initiatives, particularly in the context of payers' requirements for understanding the value of medicines. He stressed the need to address data gaps over time using RWE, noting its role as a supplement to, rather than a replacement for, randomised clinical trials (RCTs), especially in situations where such trials are impractical due to sample size constraints or feasibility issues.

Panel Discussion: The Policy Context for Real-World Evidence

The panel discussion underlined the need for cooperation with HTA bodies and Payers, particularly underscoring the importance of guidance for industry about development of RWE and the need for all parties to contribute actively to the development and review of such guidance. Stakeholders highlighted the necessity of understanding diverse perspectives, including those of patients, to ensure fair decision-making.

There were concerns voiced regarding evidence gaps during the process of marketing authorisation for Advanced Therapeutic Medicinal Products (ATMPs), emphasising the importance of maintaining a balance between traditional RCTs and incorporating real-world evidence. The focus was on ensuring reliable, high-quality data collection, especially in rare diseases, and the necessity of robust methodologies to support well-informed decision-making processes.

The role of common data models in enabling federated analysis and the review process for studies within the DARWIN-EU framework were discussed, highlighting the significance of transparency and collaboration among stakeholders. Motivating healthcare professionals to input reliable data and the crucial role of patient organisations in certifying centres were mentioned, stressing the importance of structured, interoperable data for driving evidence-based decisions.

The panellists collectively highlighted several key areas for collaboration in enhancing RWE. They emphasised the pivotal role of transparency, trust-building, and measurement methodologies as essential components for impactful cooperation. Additionally, there was a strong focus on fostering relationships between payers and the industry to enable opportunities for Value-Based Pricing through pay-for-performance models. The importance of early engagement and collaborative decision-making processes, particularly with the European Commission, was emphasised as a means to improve trust, acceptability, and overall effectiveness in shaping RWE for decision-making purposes.

LEARNINGS ABOUT REAL-WORLD EVIDENCE GENERATION AND EVALUATION – RWE4DECISIONS WORK IN 2023

KAREN FACEY

Senior Adviser HTA, FIPRA; RWE4Decisions Facilitator

Dr. Karen Facey's update on activities of the RWE4Decisions learning network highlighted the paramount importance of building trust among ALL stakeholders. In 2023 the Steering Group of RWE4Decisions was expanded to include an analytic expert, a digital health lead from OECD and two more patient representatives. Invitation only roundtable meetings, public webinars focused on implementation of the HTA Regulation and engagement in a range of conferences all facilitated dialogues about the latest methods to develop and assess RWE for HTA assessment, and post launch. Engagement with Payers was amplified this year as new methods of evidence generation are considered to support health system sustainability in light of high-cost innovative medicines.

Dr. Facey emphasised the pivotal role of transparency in their undertakings, particularly in engaging with and learning from various entities like the National Competent Authorities on Pricing and Reimbursement and Public Healthcare Payers (NCAPR). Furthermore, she underscored the network's

proactive stance in engaging with and learning from HTA bodies, citing new policy initiatives from France's Haute Autorité de Santé (HAS), and CADTH's RWE reporting framework published in June 2023. Facey's presentation shed light on the evolving landscape of guidance and learnings from regulatory bodies like EMA and, recognising their efforts while highlighting the need for coherence in approaches by different decision makers concerning RWE.

The presentation explored the growing experience in new forms post-launch evidence generation, either through bespoke national systems linked to pricing and reimbursement, like that of Valtermed in Spain, or linked to HTA recommendations through health system data collection as undertaken by NICE in England. Facey deliberated on the complexities and imperatives surrounding outcome/value-based agreements and coverage with evidence development, pointing out the necessity for early planning to enable identification and development of relevant data sources, with clear data collection protocols developed by all stakeholders that are easily accessible in other jurisdictions. The need for vigilance in data quality, with monitoring and support for data collection, and the ongoing pursuit to align these efforts across jurisdictions formed the crux of Facey's discourse. By advocating for a purpose-driven lifecycle approach to RWE, she conveyed the potential for optimising treatments, enhancing healthcare outcomes, and nurturing a sustainable healthcare continuum.

THE POTENTIAL FOR COLLECTING REAL-WORLD DATA IN EARLY ACCESS TO INFORM HTA/PAYER DECISIONS

ENTELA XOXI

Senior Researcher, Università Cattolica del Sacro Cuore Roma

CAMILLE THOMASSIN

Head of the Real-World Evidence Coordination Unit, Haute Autorité de Santé (HAS)

JO DE COCK

Former CEO, National Institute of Health and Disability Insurance, Belgium (INAMI-RIZIV)

ANDRE VIDAL PINHEIRO

Vice-President, Head of Patient Value & Access EUCAN, Global Pricing & Access, Takeda

Review of Early Access Schemes in relation to HTA

Dr. Entela Xoxi outlined how Early Access schemes are established in different health systems, presenting new research from ISPOR's Italian Chapter. Dr. Xoxi highlighted the crucial role of Early Access programs in offering treatments that are expected to have significant benefit to groups of patients that have high unmet need and no alternatives. She emphasised that Early Access provides an organised approach to provide treatment prior to standard reimbursement (and regulatory) processes and is particularly valuable in rare conditions. Dr. Xoxi stressed the importance of aligning Early Access programs with HTA evaluations and outline different types of Early Access tools, including compassionate use programs, She also discussed Italy's specific laws and requirements for Early Access, including the necessity for real-world data collection. She underlined the potential for Early Access to generate local real-world evidence parallel to international clinical trials. In Italy the collection of RWD associated with early access is written in law and occasionally is operationalised through the AIFA Monitoring Registries. In the past, several examples demonstrate how RWD collection in Early

Access has supported more rapid pricing and reimbursement decisions to enable all patients to have quicker access.

Early Access Programme in France (HTA Perspective)

Ms. Camille Thomassin provided an overview of the change in process for Early Access in France, whereby the HTA body now approves such programmes, which has been in place since July 2021. She noted that Early Access is permissible before and after marketing authorisation. Ms. Thomassin detailed the criteria for eligibility, emphasising that the product should have an acceptable preliminary benefit-risk ratio (for pre marketing early access) or favorable benefit-risk ratio (for post marketing early access), address serious, rare, or debilitating diseases, respond to an unmet need, and be considered innovative compared to existing standards of care. She discussed the process of pricing negotiations following HTA assessments and outlined the mandatory data collection requirements for Early Access programs in France. This included the collection of real-world data for each patient involved, focusing on a minimum data set covering patient characteristics, conditions of use, efficacy, safety, and patient-reported outcomes (PROMs) that is documented in a Therapeutic Use Protocol. Ms. Thomassin stressed the potential importance of these data in supporting reimbursement decisions, noting the pharmaceutical company's responsibility in data collection and payment required hospitals to support data collection costs. She also highlighted the challenges in data collection, particularly when Early Access schemes are organised close to HTA (average time between Early Access and reimbursement decision is only 9 months according to the 2-years review published in 2023), the value of patient involvement to agree relevant PROMs, and advocated for using existing data sources to simplify the process for hospitals.

Payer perspectives (Belgium)

Mr. Jo de Cock discussed Belgium's journey with Early Access schemes. He emphasised the initial aim of addressing unmet medical needs and elaborated on the establishment of Belgium's Early Access schemes a decade ago. Mr. De Cock highlighted the reform underway in Belgium to create a new system for fast and equitable access, emphasising the criteria for product eligibility and the involvement of stakeholders in decision-making. He discussed the involvement of patients in a special working group for decision-making and outlined the payment system, which remains unchanged and is a lump sum paid based on different product categories. Mr. De Cock also emphasised the creation of an Evidence Platform in Belgium that highlights the value of pragmatic, randomised trials to generate local data and this could be in Early Access, flowing into post reimbursement. Jo noted the need for investment in structured programs to collect real-world data through Early Access schemes, to inform Payers' decisions. He also highlighted the administrative burden involved in Early Access schemes and stressed the importance of addressing this issue while aiming to install and refine the necessary infrastructure.

Health technology developers' perspectives

Dr. Andre Vidal Pinheiro offered the perspectives of health technology developers. Dr. Pinheiro underscored the critical role of Early Access mechanisms in ensuring timely access to life-saving technologies for patients with high unmet needs. He acknowledged the potential for Early Access to inform HTA assessments, as outlined by the reforms in France and praised efforts in Belgium's reforms. However, he highlighted challenges, notably the short timeframe between Early Access data collection and reimbursement decisions. Pinheiro called for discussions on aligning Early Access with upcoming legislative changes like the Joint Clinical Assessment (JCA) and urged clarity in managing different standards of care across countries. He highlighted the challenging discussions underway about levels of evidence and queried how RWD from Early Access would be considered. Furthermore, he underlined the significance of Early Dialogues between manufacturers and HTA bodies, stressing the importance of establishing trust, particularly in the context of the JCA. D Pinheiro highlighted the need to bridge potential discrepancies between the PICO criteria assessed at the European level and those relevant for

member states. He proposed using a continuum of evidence to address these discrepancies and ensure alignment between different assessment criteria.

Panel discussion

During the discussion, the panellists addressed the potential value of Early Access programs to support systematic collection of RWD locally, to contribute to an important part of the continuum of evidence during the medicine's life cycle, especially in member states lacking involvement in clinical trials. The focus centred on the challenges of data collection in clinical practice, emphasising the importance of preparedness for effective data collection systems.

A question from the European Social Insurance Platform inquired about the impact of Early Access on prices, savings, and the acceptance of incomplete datasets leading to increased uncertainty. Panellists highlighted the lack of detailed information due to confidentiality agreements between companies and authorities regarding pricing and reimbursement discussions.

Dr. Entela Xoxi emphasised the complexities of Early Access in shaping pricing negotiations and informing plans for PLEG, illustrating cases where adaptive approaches were implemented based on evolving data, such as in metastatic melanoma treatments. Ms. Camille Thomassin stressed the importance of anticipating early data collection for adequate reimbursement decisions, while Dr. Andre Vidal Pinheiro mentioned the crucial role of early dialogues in enhancing reimbursement chances. He highlighted the high correlation between Early Access and reimbursement, emphasising the need to address uncertainties to define prices and the potential impact on access and solidarity among patients across different countries.

In concluding remarks, the panel stressed the necessity for anticipatory data collection for better reimbursement decisions, highlighting the need for alignment among countries, involving patients, and engaging stakeholders early on. The optimal timeframe for Early Access to generate high-quality data remained a challenge, with suggestions focusing on leveraging existing data sources, early engagement with clinicians, and patient involvement. There was also concern that the time period for data collection in Early Access would decrease given the rapid timescales of the JCA.

The discussion highlighted the critical need for early dialogue, stakeholder engagement, and anticipation of data collection to navigate uncertainties and facilitate better pricing and reimbursement decisions within the context of Early Access programs.

CLOSING SESSION: BUILDING BETTER RWE FOR DECISIONS – WHAT'S NEXT?

PEDRO FACON

Deputy CEO,
National Institute of Health and Disability Insurance, Belgium (INAMI-RIZIV)

Looking ahead to the Belgian EU Presidency

Mr. Pedro Facon, Deputy CEO of INAMI-RIZIV, highlighted the significance of informal idea-sharing among various stakeholders in decision-making processes. He noted the evolution at the European level, including the implementation of the HTA Regulation in 2025, development of the EHDS, and the General Pharmaceutical Legislation. Mr. Facon also mentioned the progress of countries to improve procedures and integrate local RWD more effectively.

Regarding the Belgian Presidency, Mr. Facon indicated that it involves regulatory processes and more political programs. He underlined Belgium's commitment to addressing unmet needs, with an intent to better define these needs. He also emphasised Belgium's active engagement in addressing medicine shortages, improving supply chain monitoring, and initiatives to incentivise and improve production capacities in Europe.

In his role representing the Payer, he highlighted the reluctance to reimburse when there is insufficient clarity or innovation in the market. Mr. Facon stressed the importance of RWE at different stages of the product lifecycle, from supporting innovation for unmet needs to aiding market authorisation, JCAs, and reimbursement procedures. He advocated for becoming a more risk-taking, strategic purchaser but emphasised the need for balance between RWE and RCTs and the importance of high-quality RWE.

Mr. Facon raised concerns about the utilisation of collected data, noting that while many data are being collected through contracts, there is a lack of capacity to effectively evaluate and exploit these data. He posed questions about the impact of leveraging RWE to improve the determination of product value and optimal reimbursement, stressing the necessity for better measurement and understanding of these impacts. He concluded by affirming Belgium's commitment to RWE4Decisions and assuring continued support from INAMI-RIZIV.

RWE4DECISIONS PRIORITIES

NIKLAS HEDBERG

Chief Pharmacist, Swedish Dental and Pharmaceuticals Benefits Agency (TLV)

MATTI AAPRO MD

Director, Genolier Cancer Center

SIMONE BOSELLI

Public Affairs Director, EURORDIS-Rare Diseases Europe

ANNA FILONENKO

Director, Real-World Evidence Scientist, Rare Disease, Pfizer

Steering Group members' reflections

Dr. Matti Aapro reflected on the progress made by RWE4Decisions and highlighted the pending questions from a clinician's viewpoint. He emphasised that while things have improved significantly in cancer treatment, challenges remain. Dr. Aapro stressed the importance of RWD collection but pointed out obstacles, such as the lack of interoperability between automated systems in different countries and languages. He highlighted the need for modern translation systems and harmonisation to facilitate data exchange. Moreover, he raised the issue of drug accessibility for patients. Despite advancements in treatments, some medicines that clinicians consider impactful are still inaccessible in certain countries. He also stressed the need for standardised methods across European countries to facilitate decision-making for HTA. He advocated for learning from studies to minimise decision uncertainty and urged a focus on utilising research funding effectively. A key point of emphasis is the substantial amount of waste within healthcare systems, amounting to 30% of all healthcare expenditures, according to OECD findings. Aapro thus called for efforts to improve healthcare systems to reduce waste and allocate resources more efficiently. Overall, he emphasised the need for continued efforts to improve data access, harmonisation, and drug accessibility for patients across various healthcare systems.

Dr. Anna Filonenko, representing the industry perspective, emphasised the paramount importance of quality in research endeavours. She highlighted a shift from an overly enthusiastic approach of analysing vast datasets to a more focused effort on conducting well-informed studies that address pertinent research questions. Quality remains the top priority for industry in conducting research. Dr. Filonenko outlined several aspects crucial for future development, notably the need for harmonised standards and methods across European countries. This consistency would benefit decision-makers, particularly HTAs, and would likely enhance and incentivise the quality of studies conducted. Additionally, she stressed the importance of continual learning from studies to reduce decision uncertainty. Dr. Filonenko emphasised the necessity of learning from instances where research funding was allocated, but the studies or evidence generated did not significantly contribute to inform decision-making. This focus on learning and refining methodologies remains pivotal for the industry's research initiatives.

As a representative of patients with rare diseases, Mr. Simone Boselli reflected on the evolution of his involvement from the earlier stages of this learning network, when TRUST4RD built a methodology to bridge gaps in rare disease research. Boselli expressed a desire for RWE4Decisions to transcend beyond just a learning network to become a bridge builder. For example, there has been significant scientific progress in developing treatments for some rare diseases, but there's still a notable drop-off in the number that successfully reach regulation and even more so in those that are reimbursed and thus

accessible to patients. RWE, in Mr. Boselli's perspective, has the potential to bridge this gap by facilitating the transformation of scientific advancements into effective therapies for patients by complementing traditional clinical research. This transformation is contingent upon considering the impact of these therapies on both patients over their disease course and the economy, and RWE4Decisions could play a crucial role in reconciling this dichotomy between scientific progress and economic feasibility.

Mr. Niklas Hedberg highlighted the critical role of RWE4Decisions in addressing HTA challenges, emphasising that while regulatory challenges exist, HTA challenges are often more complex. He echoed sentiments about Payers not always wanting to say no but wanting to spend money wisely. Looking ahead, Mr. Hedberg urged the group to consider the outcomes of their work in the long term. He highlighted that there is emerging understanding that even when the RWD to be collected are agreed upon by stakeholders in advance, there is often disagreement about its usability for decision-making purposes later on. He suggested that the community needs to collectively establish criteria for using RWE. This includes understanding if RWE should only generate hypotheses, decrease uncertainty in established knowledge, or possibly confirm or contradict data from RCTs. These different approaches, he pointed out, would lead to significantly varied outcomes in future. Mr. Hedberg stressed the importance of a unified understanding among regulators, patients, HTA bodies, Payers, and companies regarding the role and acceptance of RWE. The discussion should involve whether the RWE could alter the decision, how companies perceive the RWD, and if payers would accept the RWE to potentially influence pricing. Ultimately, the focus should be on collectively determining the standards for utilising RWE and the impact it could have on decisions across the board, acknowledging the nuances and complexities surrounding its application.

The panel's reflections centred on addressing the challenges in evidence generation for innovative treatments like ATMPs, highlighting the challenges in undertaking RCTs due to small patient populations and ethical concerns raised by investigators, especially in rare diseases and paediatric cancer treatments. Transparency is needed about the trade-offs between different approaches and clarification about what is fit for HTA/Payer purposes. Other topics included alternative evidence-gathering methods and outcomes-based payment models, necessitating discussions with payers and HTA bodies.

Panellists stressed the need for future planning, suggesting the simulation of a RWE4Decisions symposium five years ahead to gather examples of how real-world data influenced pricing decisions. Suggestions were made for aligning, anticipating, advancing, and adapting methodologies concerning RWE, particularly focusing on rare diseases to ensure timely access for patients. Additionally, there was an emphasis on all sectors learning from RWE, not only for decision-making about highly innovative medicines, but also to enhance clinical trials and improve healthcare practices. The importance of stakeholder education about RWE, and its nuances, was highlighted as crucial for the future.

LOOKING AHEAD TO 2024 – THE RWE4DECISIONS AGENDA

HANS-GEORG EICHLER

Consulting Physician,
Association of Austrian Social Insurance Institutions

In his concluding remarks, Dr. Hans-Georg Eichler delved into three pivotal reflections on the healthcare ecosystem's readiness for RWE, the role of healthcare providers in generating data, and the importance of learning from mistakes within the healthcare system. He acknowledged the varying levels of engagement among different players in the healthcare landscape concerning RWE. While industry representatives and regulators did not seem fully aligned on the potential of RWE, patients showed a considerable degree of acceptance. Dr. Eichler, speaking from his role as a payer, highlighted the need for payers to take the lead in driving this paradigm shift, underscoring their pivotal role in shaping the utilisation of RWE.

Furthermore, he addressed the issue of inadequate or missing RWD, particularly pointing out healthcare providers' contribution, especially physicians, to this problem. He emphasised the need to reshape the practice of medicine to include meticulous data entry as an integral part of healthcare provision. Dr. Eichler emphasised the societal value of accurate data collection and the potential impact it could have on informed decision-making and patient care.

His personal reflection focused on the critical aspect of learning from mistakes within the healthcare system. Dr. Eichler drew parallels between individual learning and the healthcare sector, stating that a system unable to learn from past experiences could be considered 'stupid'. He criticised the existing legal barriers that inhibit the secondary use of data for learning purposes within healthcare. Instead, he advocated for a shift in attitude toward fostering a culture of learning within the healthcare system, emphasising the importance of this approach for better decision-making and improved patient outcomes.

RWE4Decisions REAL WORLD EVIDENCE

RWE4Decisions is a 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 is led by a multi-stakeholder Steering Group and contributors include HTA bodies and payers, regulatory agencies, patient groups, clinicians, registry-holders, data analysts, industry and academic experts/researchers.

For further information and to watch the recording of the Symposium, visit our website:
https://rwe4decisions.com/event/real-world-evidence-over-the-medicines-lifecycle-to-inform-hta-payer-decisions/

WE WANT TO HEAR WHAT YOU ARE DOING TO PROGRESS LEARNINGS ON THE USE OF RWE!

Contact us at secretariat@rwe4decisions.com if you would like to join the RWE4Decisions Learning Network.