SUMMARY REPORT

RWE Case Studies for HTA Decision-Making

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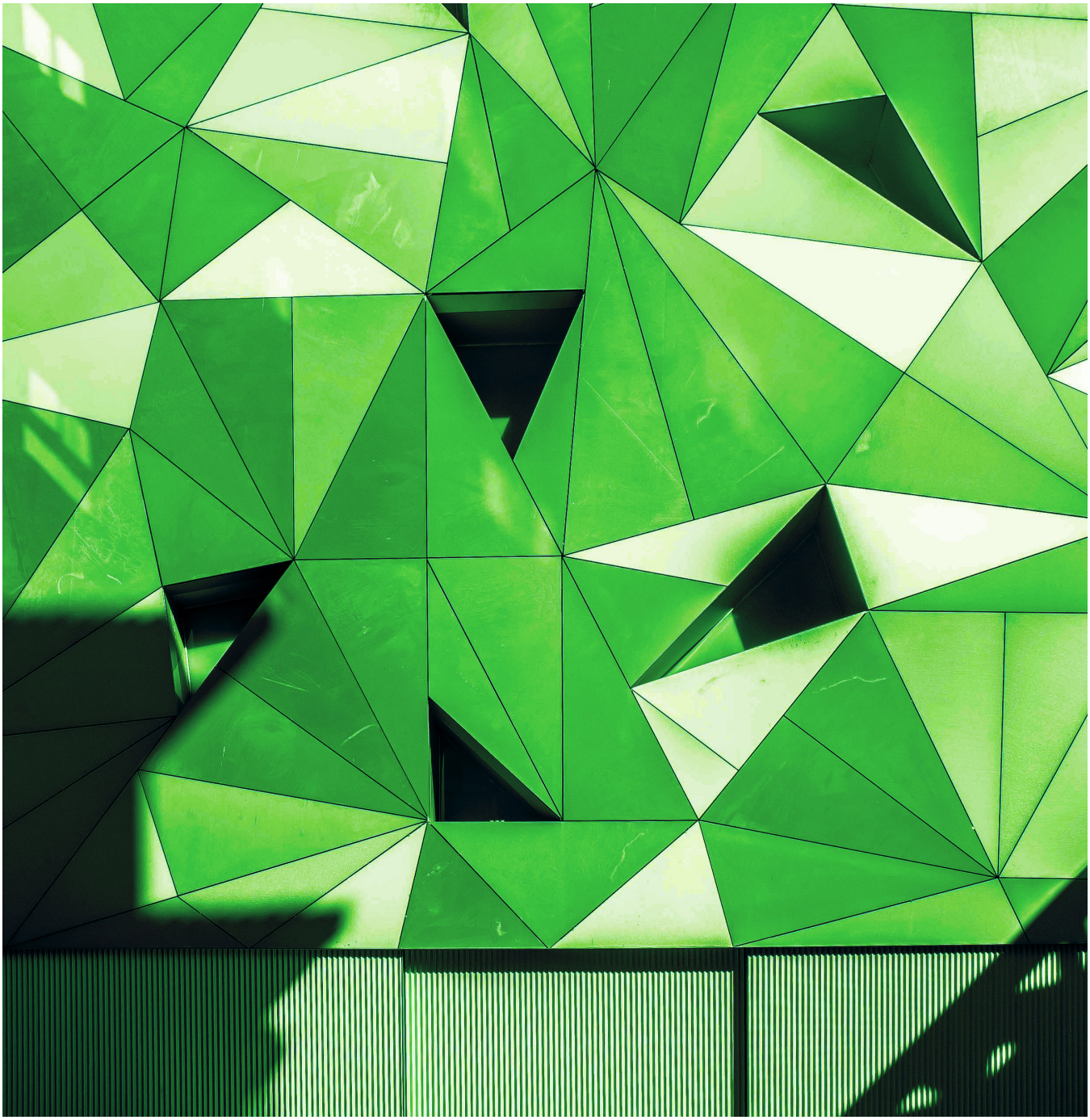

GETREAL INSTITUTE'S SHARED LEARNING FORUM

GETREAL INSTITUTE | PUTTING REAL WORLD EVIDENCE INTO PRACTICE

Background

In the dynamic landscape of healthcare, the pursuit of well-informed, evidence-based decision-making is more imperative than ever. The GetReal Institutes' "Shared Learning Forum" aims to cultivate the development and dissemination of generalisable learnings surrounding the opportunities and limitations of using Real-World Real-World Data (RWD) / Real-World Evidence (RWE) in defined decision contexts, while drawing insights from case study applications of RWE in supporting regulatory, reimbursement, and payment decisions. The Forum aspires to reshape the paradigm of healthcare decision-making where RWD/RWE would be accepted as evidence with the potential to improve patient outcomes and accelerate acess to medicines.

GetReal Institute brings together a diverse spectrum of stakeholders, including Health Technology Assessment (HTA) bodies, Regulatory authorities, industry experts, and patient advocates. For the first iteration of the "Shared Learning Forum", we were pleased to introduce general research inquiries stemming from a genuine RWE case study using registry data, as a starting point of the discussion, paving a way forward for informed decisions about treatment effectiveness, safety, and reimbursement.

Session Highlights

A COMPREHENSIVE OVERVIEW OF EXISTING REGISTRY DATA

- The European Federation of Pharmaceutical Industries (EFPIA), provided a thorough analysis of existing registries, highlighting the imperative acceptance of registry data as evidence for improved patient outcomes.
- Guidelines and recommendations presented include: the EUnetHTA REQueST (Registry Evaluation and Quality Standards Tool), PARENT the Cross Border PAtient REgistries iNiTiative by the Joint Action EU Health programme 2008-2013, Canadian Agency for Drugs and Technologies in Health ("CADTH"), in collaboration with Health Canada and the Institut national d'excellence en santé et en services sociaux ("INESSS"), EMA Guidance on Registries to Support Regulatory Decision-making for Drug and Biological Products Guidance for Industry, FDA Assessing Registries to Support Regulatory Decision-Making for Drug and Biological Products Guidance for Industry, and the 4th edition of Patient Registries for evaluating patient outcomes, as user guide, by the Agency of Healthcare Research and Quality (AHRQ).

OVERVIEW OF A GENERAL RWE CASE STUDY USING DATA REGISTRY TO CONFORM TO HTA REQUIREMENTS AND INFORM TREATMENT EFFECTIVENESS, SAFETY, AND DETERMINE REIMBURSEMENT DECISIONS

- An overview of a generalised Real-World Evidence (RWE) case study, presented by the European Federation of Pharmaceutical Industries (EFPIA), focused on using registry data to meet Health Technology Assessment (HTA) requirements.
- The case involved "Product X" seeking HTA approval, revealing uncertainties from pivotal trials. EFPIA aimed to showcase the current approach to leveraging registry data for HTA, acknowledging potential variations across countries. Despite being largely hypothetical, the case highlighted challenges in initial study design.
- A Product Registry was established to address uncertainties, focusing on short to long-term effectiveness and safety data, leading to conditional reimbursement decision. Upon reassessment, the HTA body considered the real-world observational data consistent with trials, leading to a favourable opinion for reimbursement maintenance, with a recommendation for further follow-up due to data immaturity.

INSIGHTS FROM THE EXPERT PANEL

- Analysis by the European Organisation for Rare Diseases (EURORDIS) provided valuable insights into the complexities of the case study, particularly emphasizing a multi-stakeholder perspective, and the need for detailed data collection. Challenges with registry data were also underscored, including governance issues, variations in treatment approaches, and the combination of disease and product registry aspects. Patient community engagement and collaboration between researchers and patient organizations to gather input for assessing the real impact of treatment on patients' lives was also underscored.
- The Dental and Pharmaceutical Benefits Agency (TLV) discussed the Swedish experience, highlighting the significance of utilizing registries for data collection despite limitations in the number of patients and practitioners. Using the Swedish Health System as an example, he emphasized the benefits of registries for tracking consumption patterns, especially with advanced therapies. Two specific cost scenarios were presented, illustrating the impact of prolonged dosing intervals on cost savings and increased costs due to assumptions about prolonged half-life. Challenges with data completeness, especially for one-off treatments, were acknowledged, prompting the need for modelling assumptions.
- The National Institute for Health and Care Excellence (NICE) discussed the scenario as familiar in the UK highlighting the established practice of managed access agreements, with conditional recommendations dependent on evidence generation. The supportive role of Real-World Data was emphasized, contextualizing, and providing reassurance for trial outcomes. Challenges of multiple reappraisals were raised, considering potential burdens on Health Technology Assessment (HTA) agencies, and the use of patient registries

to provide comprehensive real-world comparative effects estimation alongside trial data as a more holistic view of treatment outcomes in real-world settings.

- The European Medicines Agency (EMA) offered concise yet insightful remarks during the discussion, highlighting important considerations and potential challenges when setting up disease registries and registry-based studies, especially in the context of rare diseases. Seeking early scientific advice from regulators and HTA in the design of registries and registry-based studies was recommended to help anticipate and plan evidence needs.

MODERATED DISCUSSION WITH QUESTIONS ADDRESSED TO THE AUDIENCE

In the discussion, designing registries for Health Technology Assessment (HTA) excellence was highlighted, emphasizing considerations like data suitability, governance, and stakeholder engagement with a focus on anticipating evidence gaps.

The timing for data submission was addressed, stressing the importance of early investment in existing registries for meaningful insights. Challenges in registry implementation and generating data quality, patient consent, and the necessity for multi-stakeholder collaboration, were acknowledged. The conversation also touched on crafting registries for HTA excellence, involving various tools, and the need of patient involvement. Time constraints for data submission and reimbursement impact were discussed, with an emphasis on early investment in existing registries. Overcoming challenges in registry implementation, including real-world evidence through initiatives like DARWIN EU, was also explored.

The overall consensus highlighted the need for strategic planning, early engagement, and multi-stakeholder collaboration to enhance the effectiveness of health registries in informing decision-making processes.

Stakeholder Recommendations

1. REGISTRY ESIGN

a. Emphasise strategic design considerations for crafting registries for Health Technology Assessment (HTA) excellence and underscore the importance of early engagement with diverse stakeholders for improved decision-making in healthcare interventions.

b. Prioritise data source agnosticism, good data provenance, and effective governance. Engage patients and clinicians in dataset design to ensure relevance and fitness for purpose.

2. REGISTRY ADOPTION

a. Promote widespread understanding of registry data as valuable evidence for health outcomes and healthcare decisions.

b. Utilise registries for historical data collection, ensuring meaningful insights into treatment effectiveness and facilitating regulatory and reimbursement decisions.

3. MULTI-STAKEHOLDER COLLABORATION

a. Facilitate multi-stakeholder collaborations to address challenges related to data quality, implementation, and ethical considerations.

b. Address perceptions and misconceptions regarding registry data utilisation.

4. GUIDANCE & STANDARDS

a. Champion the adoption of international guidelines and standards, leveraging comprehensive frameworks, to ensure transparent and globally aligned regulatory and HTA decision-making.

b. Identify challenges and provide support towards implementing and adhering to standards.

5. FUNDING & STABILITY

a. Support early investment in patient registries and their development to address evidence gaps in healthcare, and advocate for incentives to streamline data collection processes.

b. Recognize the dynamic nature of registry implementation and address challenges related to data quality, consent for secondary data use, and ensure adaptability to evolving healthcare landscapes.

These recommendations collectively aim to foster a collaborative, evidence-driven approach to registry development and utilisation, ultimately contributing to more informed decision-making in healthcare.