EVENT REPORT

REAL WORLD EVIDENCE
RWE4Decisions
PUBLIC WEBINAR SERIES

Supporting HTA/Payer decision-making: Spotlight on national health data initiatives from Finland, Spain and France

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This RWE4Decisions webinar explored the enactment of national data initiatives in Finland, Spain and France that could support HTA/Payer decision-making. The importance of engaging all partners in the development of health data ecosystems, governmental support, capacity development and improving health data literacy were identified as key factors.

Finland has world-renowned health and social care data arising from sources such as administrative records, national registries, biobanks, laboratory data, primary care records and more recently hospital data lakes. All these real-world data (RWD) can be linked via a unique citizen identifier within a secure data infrastructure that aims to collect data once and use it many times. The Finnish ecosystem for safe and effective use of data has required three elements (1) national legislation about the secondary use of health and social care and biobank data, (2) public investment and (3) clinical support.

The legislation ensures strict governance processes, operationalised by Findata who issue permits enabling bona fide use of data within a secure and audited environment, whilst Fingenious® governs use of data from biobanks. This ensures that the rich data resources can be used appropriately for research, create a learning health system and improve patient outcomes. These real world data could be a particularly valuable resource for HTA and pricing and reimbursement decisions, but the wide range of actors involved in these negotiations need to align their requirements and engage with the data authorities to explain their needs.

Spain has established a new information system, Valtermed, to determine the real-world therapeutic value of medicines and inform pricing and reimbursement negotiations. Currently, the system includes 20 medicines which have a high health and economic impact on the national health system and for which major clinical uncertainties exist. Collection of data per disease/condition is agreed by consensus among the experts from the Spanish regions, national professional societies and the Marketing Authorisation Holder. This is published in a protocol that is available in English[1].

All protocols include a minimum set of real-world effectiveness and safety assessments, plus bespoke data collection for the medicine at specified timepoints, based on the clinical uncertainties and reimbursement conditions (such as mixtures of discounts and pay for performance, pay backs, pay per milestone, loading dose free, etc.) This then leads to development of an electronic data collection template that is bespoke for each medicine but is governed within the Valtermed system. Data can be easily extracted, to review progress and alter the reimbursement conditions.

[1] https://www.sanidad.gob.es/profesionales/farmacia/valtermed/home.htm

The Valtermed system supports collection of good quality data that not only enables application of more complex outcomes-based reimbursement schemes and determination of budget impact, but also creates good real-world data for other purposes such as creation of a comparator for new drugs in the same condition. Reports are publicly available and thus build knowledge internationally. Valtermed is a modular system and so can be developed with experience. One key improvement will be the addition of a quality of life module soon. Feedback has also identified concerns from clinicians about the increased workload created by data entry and this remains a challenge, but the aim is to address this through linking Valtermed with electronic medical records.

In **France**, patient involvement in the national HTA process has improved in recent years. This means that deliberations about added therapeutic benefit can be augmented to include patients' perspectives about the impacts on their well-being and ability to play an active role in society. The value of these real-life inputs has been recognised and investment is being made at the national level in France to ensure patient-reported experience/outcomes measures will be available to evaluate health system performance and improve clinical care.

One important measure of health system performance is that of time to treatment availability after marketing authorisation. France is often shown as being in the middle of EU countries in terms of performance, but this does not take account of the Autorisation Temporaire d'Utilisation (Temporary Authorisation for Use) process. This ATU process, organised by the national regulator, has been in place for almost 20 years to support early access to pre-authorised highly innovative medicines for severe or rare diseases with high unmet need and no alternatives. The cohort ATU (cATU) can be requested by the Marketing Authorisation Holder to enable a group of patients to access the medicine according to a well-defined protocol. This generates real-world evidence to determine real-life effectiveness and determination of the added medical benefit (Amelioration du Service Médical Rendu, ASMR), for use in pricing and reimbursement negotiations. In summer 2021, reforms were enacted to rename the cATU as the "Early Access Programme", governed by the HTA body, Haute Autorité de Santé (HAS). This requires data collection via the protocol to have started before the application has been approved and then provides RWD that is accessible to the HTA body to inform its decision-making.

RWD is clearly valuable for pricing and reimbursement decisions to validate, or invalidate, claims of benefit made in small uncontrolled clinical trials, but as there are many methodological and operational challenges, randomized-controlled trials are still preferred. But this is not the only use of RWE, it also has an important role to play in patient safety and improving the quality of care – ensuring equitable access of patients to approved treatments, optimizing the patient journey, creating efficiencies in delivery of care and closing the evidence-practice gap.

A lively panel discussion with the audience considered issues including:

- The most important aspects of the proposed legislation for the European Health Data Space Regulation that had just been released;
- The balance between avoiding over-legislation and considering the differences in European Member States' capacities regarding data management;
- The potential of using anonymised health data for research purposes without GDPR-consent requirements;
- Approaches to reduce the burden of data collection on clinicians and the potential to promote data collection as a means to improving individual patient care and clinical practice;
- The importance of public involvement to agree processes for data collection and data governance.

The panel concluded that many national advances are being made in developing data ecosystems for health and social care. These have important potential not only for all the actors involved HTA/payer decision making, but also by health services to improve system performance and clinicians to optimize patient care. Such health (and social care) data ecosystems require national legislative support to ensure all relevant decision makers can access data in a secure environment, long-term investment in infrastructure and human resources, and education to improve citizens' health literacy.

RWE4Decisions REAL WORLD EVIDENCE

RWE4Decisions is a multi-stakeholder group, 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 (INAMI-RIZIV) and contributors include HTA bodies and payers, regulatory agencies, patient groups, clinicians, industry and academic experts/researchers.

For further information and to watch the recording of the webinar, visit our website at https://rwe4decisions.com/event/public-webinar-series-national-health-data-initiatives-in-finland-spain-and-france/

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