

Generating Real-World Data and Using Real-World Evidence for HTA Purposes

Updates since the 2019 HTAi Global Policy Forum

Panel Session at the HTAi Annual Meeting in Cologne, June 2019

A panel session in Cologne was moderated by Laura Sampietro-Colom (Global Policy Forum Chair) and opened by Wija Oortwijn (Global Policy Forum Scientific Secretary) with a summary of the discussions that had taken place during the 2019 HTAi Global Policy Forum (GPF). Wija's slides are available on the HTAi website [here](#). Reflections were then delivered from different perspectives by Andreas Hager (invited keynote speaker to the 2019 GPF, patient perspective) and GPF members: Wim Goettsch (ZIN, Netherlands, HTA agency perspective), Marguerite Koster (Kaiser Permanente, USA, payer perspective) and Alicia Granados (Sanofi, industry perspective). Following the presentations, and during the audience Q&A and discussion, an interactive app (Sli.do) was utilized to gather audience opinions on four pre-prepared questions.

As previously described, the 2019 GPF convened 73 representatives from HTA agencies, industry, the HTAi Board, and invited keynote speakers in Barcelona, Spain in January 2019. The objective was to discuss the challenges and opportunities for using Real-World Evidence (RWE) in the context of HTA from a policy perspective. The discussions were centered on four themes: *quality and acceptability, governance and accountability, transferability, and informing decision-making*. One key theme that was apparent



throughout the 2019 GPF was that the tide of change is approaching; regardless of the HTA community's readiness, regulators and other relevant organizations in the field have already signaled that there will be an increased use of RWE, so the time to act is now.

A key message coming from the 2019 GPF that was strongly reiterated during the panel session was that increasing trust, building relationships and better collaboration is critical to embrace and act upon the tide of data change. However, it was noted at the panel session that, in reality, there are actually few incentives for industry and others (particularly payers) to actively collaborate and work together. Furthermore, where there are fledgling examples of public-private partnerships these are often relatively short and they lack stability. Practical ways to address these issues that were suggested included ensuring industry to provide equitable access to products and that guidelines, and clarification on relationships are developed (again, here trust will be key). Considering the problem in more "bitesize, manageable pieces" could also be valuable.

However, to do this, we – as a community - must prioritize and agree what Real-World Data (RWD) and RWE is best for (i.e. which questions and parts of the HTA process will benefit the most from RWD).

As was discussed at the 2019 GPF in January, it was acknowledged that the future is already here and we should be starting to actively work today to move projects into use in real-world decision-making. Additionally, it was reiterated that the concept of RWE being used in HTA is not new (for example economic models are typically built using RWE). The panel and audience highlighted there are some good examples – for example in Catalonia where a law has been passed to implement an interdepartmental plan for public health (PINSAP) that aims to facilitate patient level data collection and analysis; or in Scotland where robust, pragmatic data are being routinely collected in primary care to enable better decision-making (Scottish Primary Care Information Resource, SPIRE). The key message from the panel session was still that the use and acceptability of RWE for HTA all depends on the level of uncertainty that a decision-maker is willing to accept. If decision-makers are traditionally more risk averse, and if this needs to be changed to increase the acceptability of RWE, then trust is again paramount.

Increasing use of RWD and RWE also depends on factors such as the development of methodological standards. When asked whether it is necessary to develop a GRADE-like assessment tool for RWD/RWE, 91% of the panel audience responded, yes, it is necessary. However, when asked if there are any differences in collecting RWD on different types of technologies (drugs, devices, diagnostics), the panel audience was split with 45% saying that there are differences, and 50% saying that there are not.

At the end of the panel session, the audience was asked if they believed whether RWD should ALWAYS be used to generate RWE for use in HTA. A total of 65% said no, RWD and RWE should not always be used for HTA (this changed from 55% saying no, with 12% undecided, at the beginning of the panel session). This implies that the use of RWD and RWE in HTA is still not determined or clear cut and that there is still work and collaboration required in this area. These sentiments are echoed in the selection of the third plenary session at the upcoming HTAi Annual Meeting in 2020 in Beijing, on **Surfing Data Tides: HTA in the Era of Real-World Evidence and Artificial Intelligence**, where the ongoing debate on this topic will continue.

RWE Impact in the Field

Featured impact story: CADTH

CADTH highlights two initiatives showcasing RWE impact:

RWE and HTA

1. Developed and executed a multi-stakeholder RWE Workshop
2. Governance and collaboration developed
3. Produced RWE framework
4. Engagement and Communication framework

CAR T and HTA

1. Developed a Coordinated Approach to the Implementation of Gene Therapies in Canada
2. Coordinated a Canadian CAR T-cell Summit to discuss system capacity and access, clinical and ethical decision making, decision making parameters, implementation and pricing
3. Created an Ad Hoc Policy Committee to discuss issues with jurisdictions, price negotiation, coordinated approach to review and recommendation release



Brian O'Rourke
President and Chief Executive Officer