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Burning Issues in Health Technology Assessment and Policy Making: What's Keeping Senior Health Technology Assessment Users and Producers up at Night?

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A key working session, held as part of the Health Technology Assessment international (HTAi) Global Policy Forum meeting asks members to share "What's Keeping Me Up At Night." Members—senior thought leaders from health technology assessment (HTA) agencies, payer organizations, industry, and the HTAi Board—share without fear or favor the thorny issues related to HTA that are challenging them now or likely to do so in the near future. This article contains a reflection on the discussions at this session over the last 2 years and focuses on the recurrent and repeated themes: internal and external stakeholder involvement in HTA processes; globalization of HTA and the future of HTA (namely innovative technologies, tide of data and the "war for talent"). While the aim of these informal sessions is not to produce solutions, it reinforces the importance of developing a truly multi-stakeholder HTA community with working relationships built on mutual trust and long-standing engagement.

Perspective

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The Health Technology Assessment international (HTAi) Global Policy Forum (GPF) is an annual event that provides a unique opportunity for senior representatives from public and private sector organizations using or producing health technology assessment (HTA) for strategic discussions about the present state of HTA, its development and evolution, and the implications of HTA for healthcare systems, industry, patients, and other stakeholders. The aim of the GPF is to provide a "safe harbor" environment where senior representatives can engage in conversation informed by the perspectives of their different organizations without the constraints associated with the discussions of specific products or organizational policies.

The GPF gathers once a year to engage in discussion and debate around a policy topic of shared interest. Previous examples include the use of real-world evidence (RWE), horizon scanning, and value frameworks. In addition, for the past 4 years, an adjunct working session titled "What's Keeping Me up at Night" has been a feature of the first day of the HTAi GPF. The objective of this session is to identify pressing current "burning issues" that GPF members are facing in their day-to-day work in relation to HTA and policy decision making, which may or may not be directly related to the main topic to be addressed at that particular forum meeting.

During the What's Keeping Me up at Night session, the GPF members are split into discussion groups. The intent of the session is not to problem solve, but rather to provide a neutral venue where current or potential future burning issues can be frankly discussed. It is interesting to note that when this session was first introduced, the discussions were held in groups categorized by the "not-for-profit" or "for-profit" status of each attendee to allow for open sharing and discussion among peers. However, at the request of the GPF members, these sessions are now held with discussion groups with mixed membership. This gradual shift to mixed groups is indicative of the building levels of trust and working relationships that arenas like the HTAi GPF can foster.

This perspective piece is a reflection on the 2018 and 2019 What's Keeping Me up at Night sessions; it is not intended to be a comprehensive review of all items that were discussed, and respecting the Chatham House rule (1) under which the GPF meetings are conducted, no statements are attributed to any individual member. In both 2018 and 2019, there were three broad areas of discussion: internal and external stakeholder involvement in the HTA process, globalization of HTA, and the future of HTA, as described further below.

Internal and External Stakeholder Involvement in HTA Processes

The term "internal stakeholder" refers to participants directly involved in the HTA process, such as the HTA workforce, patients, and clinicians. There is an increasing level of direct

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participation of these types of stakeholders in HTA processes (e.g. topic selection, preparation of assessments, committee deliberations, production of guidance/recommendations). While most would agree that greater inclusiveness on the part of HTA is a good thing, there are also concerns regarding how to identify and meaningfully include all relevant stakeholders, who is the best representative of each stakeholder type, and when they should be included. In addition, increasing involvement of stakeholders can lead to the involvement of a relatively small, HTA-educated, body of individuals (such as selected patient representatives) who can become stretched. There are challenges therefore in determining transparent and consistent processes and methodologies for ensuring that meaningful input is captured and appropriately considered during the HTA process (2).

The term "external stakeholder" refers to those who traditionally do not directly input or interact with the HTA process although they are affected by the results; it is acknowledged that "external" stakeholders—such as the general public, regulatory agencies, political bodies, and the judiciary (the latter particularly in Latin America) do still have varying levels of input, albeit indirectly. The involvement of external stakeholders (and processes such as legislative processes) has the potential to derail the HTA process and challenge the autonomy of the decisions or recommendations made. Discussions on these issues revealed that many felt that concerns—such as decision making not being evidence-based in accordance with the findings/recommendations from an HTA process—are largely borne of ignorance of HTA, and will continue unless there is an improved knowledge and understanding of the importance of HTA by all external stakeholders. Without understanding from at least the relevant external stakeholders, there may even be threats to the sustainability of HTA as a concept given the ongoing budgeting constraints and challenges faced by healthcare systems around the world.

Many in these sessions felt that this is leading to a divide between HTA assessment and subsequent adoption and implementation in healthcare systems. This appears to be coupled with a departure from the conduct of comprehensive, "full," HTA (i.e. including the ethical, legal, safety, and social elements) with a limited focus on assessing the clinical and cost-effectiveness of technologies simply to inform pricing decisions. Globally, there is often a lack of payer literacy with HTA outputs, which leads to limited translation of HTA results into coverage decisions. This is combined with a seemingly increased willingness to accept uncertainty in the regulatory space (i.e. limited data sets from pivotal clinical studies, use of surrogate end points) for the purpose of speeding marketing authorization, and the possible misuse of social media and "fake news" by various stakeholders. These factors are all creating greater pressures for the HTA community to respond to; the balance of including and informing all relevant stakeholders while ensuring HTA remains objective, relevant, and timely is an ongoing challenge.

Globalization of HTA

During the 2018 and 2019 sessions, a good portion of the discussion focused on the current and future potential for changes in the context of globalization of HTA. The changing role of HTA in Europe after 2020 (with the European Commission striving to increase and even mandate collaboration (3)) has been a recurrent theme, and the possible impact of HTA elsewhere is of great interest to GPF members. Observing what is happening in Europe and considering what other countries can learn from this (e.g. shared approaches and collaboration) will be an important step in the process.

It was noted however that there are still divergent views, even within Europe, with regards to a common HTA approach across countries; collaboration is challenging. For example, it may or may not be appropriate to simply replicate HTA structures across countries, given differences in health system dynamics, reimbursement processes, and local implementation. In addition, there are cultural differences in the perspectives employed in an HTA assessment (i.e. value frameworks), as societal values, acceptance of risk, and willingness to pay for improvements in health vary by definition across nations—all of which lead to challenges in working across jurisdictions. The question of whether and how evidence, HTA processes, and HTA outputs can be transferred across settings remains a persistent one.

The challenges highlighted are of course countered by the potential efficiency gains of collaborative or joint action efforts where appropriate (e.g. on the clinical aspects of HTA). This is supported by an increasing body of work on what characterizes "good practices" in HTA (4). This is particularly important for emerging HTA markets (e.g. Asia and more recently Africa) who have the opportunity to take guidance from more mature HTA systems rather than developing governance, structure, and processes "from scratch" (5).

The Future of HTA

There are many current and future challenges for traditional HTA methodology and processes. These include:

(a) "Transformative"/"disruptive"/"regenerative" health technologies (i.e. technologies that have the potential to profoundly change the healthcare sector through unique delivery systems, curative potential, or other mechanisms);
(b) the rise of RWE, artificial intelligence, combination technologies, eHealth, and other digital health interventions;
(c) precision medicine and increasingly clinically complex and demanding patients.

These factors need to be carefully considered by the HTA community. New methods for assessment, including in both the regulatory and the postmarket space, and novel trial designs are also undergoing an unprecedented rate of evolution. This is combined with the increase in off-label use and an era of rapid indication change/expansions. This tide of change is already upon the HTA community and cannot be avoided.

A critical issue also with the prevalence of innovative therapies and important in considering the future of HTA is that affordability and sustainability of healthcare systems are increasingly influencing and driving the HTA process. This is of particular importance in middle-income countries, where access to innovative therapies is often challenged due to limitations on capital and related expenses. The definition of what is truly meant by "breakthrough" and whether "unmet need" can be better quantified should also be considered in this context. There are also other pertinent challenges for the future of HTA, including the impact of aging and clinically complex populations on healthcare systems, how evidence on end-of-life care should be assessed, and how HTA methods/processes should be used outside of drugs and devices.

All of these issues that are either already present or looming on the horizon are all an even greater issue and will likely be

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magnified by what was described as GPF members as the "war for talent." The advent of new technologies to be assessed, and also new technologies and methods to be used as part of the assessment, will likely necessitate a raft of new skills that are not yet commonplace in the communities of HTA producers ('doers') and consumers ('users'). HTA practitioners will therefore need to be upskilled, as will certain stakeholders to ensure continued meaningful engagement. Given that HTA and industry may in fact compete for the same skill sets, there indeed may be a "war" for the right individuals. Much debate is ongoing about whether the existing HTA methods are satisfactory for the different types of new health technologies (e.g. with the advent of gene editing and regenerative medicines) and also whether we have the ability and skills to deal with the larger volume of data (e.g. how can this be processed and analyzed most usefully). This is of course all balanced with the need for speed and timeliness of HTA reports (noting also the increasing pressures on the HTA process already described in this article).

Moving Forward

While these sessions are not designed to explicitly produce solutions, there were some suggestions that were noted and reiterated by GPF members. For example, it was noted that pragmatic resourcing, widespread training opportunities (for HTA agencies, industry, and others), and possible specialization of HTA agencies (e.g. by type of technology) could all be explored. Additionally, the definitions of best practices in transferability should be developed to guide increased harmonization and cross-jurisdiction collaboration.

HTAi will continue to take forward some of the issues raised, for example, the next GPF will focus on "Principles for Deliberative Processes in HTA" (26–28 January 2020 in New Orleans, USA) (6). Additionally, the issues raised in the What's Keeping Me up at Night session were reviewed and directly informed the plenary themes for the 2020 HTAi Annual Meeting (20–24 June in Beijing, China) (7). This will encourage further debate and scientific development in these key areas for HTA that are, or will be, of utmost relevance to all in the HTA community. Perhaps most importantly, these informal sessions will continue to reinforce the most important aspect of multi-stakeholder HTA—that issues, even if difficult and subject to opposing views, are best considered first in conversation with each other.

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