Commentary

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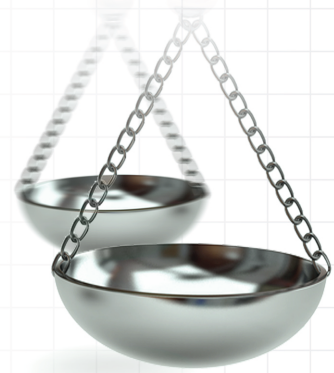

As health technology assessment evolves so must its approach to patient involvement

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> "we need to rethink how we can invest in patient involvement to make the best use of all forms of research methodologies and collaborate across borders to develop an understanding of patients' perspectives and experiences that may not be health technology specific."

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Before reflecting on patient involvement in health technology assessment (HTA), we need to understand the specific context of HTA and how it has evolved.

HTA: born in the USA

The structure and form of health systems differ around the world, but most have the same goals to organize their services efficiently to deliver effective, safe, person-centered care in a timely and equitable manner for the population they serve [1]. They also all have to make difficult decisions about how to use their limited financial resources and consider whether they require copayments from their users for services and treatments. Daniels and Sabin [2] stated that such resource allocation decisions in healthcare are "*rife with moral disagreements and a fair, deliberative process is necessary to establish legitimacy and fairness of such decisions*". These decisions have become even more challenging in recent years as scientific advancements lead to development of highly innovative and potentially curative therapies that are available at high cost.

But discussions about wise investment in healthcare resources are not new. In 1975, the Office of Technology Assessment (OTA) established a health program and began assessing 'medical technologies' – diagnostics, implantable devices, vaccines, surgery, medicines and interventional procedures [3]. It used 'Technology Assessment' (TA) to systematically examine the short- and long-term social consequences (e.g., societal, economic, ethical and legal) of the use of technology, considering unintended, indirect and delayed social impacts, including implications for:

- Patients;
- Patients' families;
- Society as a whole (environmental impacts, ethics and cultural values);
- Medical care system;
- Legal and political systems;
- The economy.

OTA's TA manual [3] included questions about the impacts on patients and their families that are as relevant today as they were four decades ago, relating to quality of life, psychological issues, reversibility of technology, ethical considerations, impacts on family life, living circumstances and costs to patient and family.

Full HTA: evidence to determine all impacts

In the 1980s, Denmark and Sweden learned from the USA and established national organizations to undertake systematic assessments of all forms of health intervention (including 'medical technologies', educational programs and organization of care). Definitions of HTA emerged that drew on OTA's ideals of a systematic approach to

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considering all the implications of using a health technology. These have stood the test of time as shown by the European Network for HTA's description of HTA as "*a multidisciplinary process that summarizes information about the medical, social, economic and ethical issues related to the use of a health technology in a systematic, transparent, unbiased, robust manner*" [4].

HTA seeks to inform policy decisions by using robust scientific processes involving critical assessment of all relevant international evidence by methodological experts. This can be seen in the Danish HTA handbook [5] that was first published in English in 2001 and presented a comprehensive model of HTA based on:

- Clinical effectiveness;
- Cost–effectiveness;
- Organizational issues;
- Patient aspects.

The handbook presented methods to systematically review literature and undertake primary research to understand each of these areas including qualitative methods, survey methods and analysis of registries. It was expected that this work would be undertaken by expert staff in an HTA body and could often take several years. However, the synthesis of all these elements was deemed necessary to formulate a sound basis for decision making.

Over the next decade, collaborative work among HTA bodies in the EU delineated more essential elements for HTA as outlined in the nine domains of the HTA Core Model® [6]:

1. Health problem and current health technologies;
2. Description/technical characteristics;
3. Safety;
4. Clinical effectiveness;
5. Costs/economic evaluation;
6. Ethical analysis;
7. Organizational aspects;
8. Patient and social aspects;
9. Legal aspects.

The HTA Core Model also included comprehensive methodological guidance for the study of each domain that was intended to guide HTA staff and allow joint working on HTA report development across countries.

These types of HTAs that rely on systematic methodological approaches to assess each of the impacts of a health technology and which explicitly include patient aspects, have been termed 'full HTA'. Unfortunately, they are rarely undertaken today.

HTA to inform reimbursement: the demise of patient aspects

Alongside the collaborative work on HTA methodology in the 2000s, decision makers were faced with the challenge of deciding which pharmaceuticals to reimburse amidst high profile campaigns from patients demanding access. HTA was seen as a robust process to help determine the added value (or clinical benefit) of the medicine compared with the current standard of care and in some jurisdictions its value for money. However, as EU law required decisions to be made soon after their marketing authorization approval, HTA needed to be faster. This led to a move away from HTA researchers undertaking full HTAs. Instead, health technology developers were asked to submit evidence relating to clinical and cost effectiveness, which was critically assessed by expert staff, before wider consideration by an appraisal or decision-making committee.

The structured assessment of the wider implications of using a health technology was lost [7]. The consideration of wider impacts was left to the appraisal committee.

Appraisal committees vary in composition, but generally include representatives of the payers and providers of healthcare in the region and in some cases may include other stakeholders. They need to exercise value judgments about the uncertainties relating to estimates of treatment effect and value for money. These are becoming more challenging as expedited regulatory approvals, new treatments for rare disease and highly innovative products mean that health technologies come to market with a small clinical evidence base. Appraisal committees spend time ensuring that the analyses presented to them are based on assumptions that fit with their understanding of the

health system and the condition in question (population to be treated, place in pathway, comparator, relevant outcomes, etc.). Little time is spent on the wider impacts beyond clinical and cost effectiveness.

Patient involvement could help appraisal committees resolve uncertainties providing unique insights into living with the medical condition, how the clinical trials reflect local practice, the benefits and challenges with current treatments, unmet needs and what would be deemed as added value in a new therapy. Their involvement could of course also bring an understanding of the wider impacts of using a health technology.

What is patient involvement in HTA?

In 2015, the Ontario Health Technology Advisory Committee determined that patient involvement could contribute to democratic, technocratic, scientific and instrumental goals in HTA [8]. It recommended that each HTA organization should define its goals of involvement and tailor (participation and research) approaches accordingly. However, before considering goals and approaches for patient involvement, we first need to think about what we mean by 'patient' and 'involvement' in the HTA context.

In HTA, we use 'patient' as a broad ranging term that covers anyone who has direct experience of living with the condition being studied in the HTA or who may be eligible to receive the technology (e.g., specific members of the public who might be invited for vaccination or to undertake a diagnostic intervention). This can include individuals who have had or have the condition, informal care givers (sometimes called carers) and patient group representatives, but their perspectives are different and should be differentiated.

Internationally, it has been recognized that patients have unique experiential knowledge and to elicit this knowledge and optimize its use, we need their involvement in HTA in two distinct but complementary ways:

- Patient participation in the HTA process;
- Research into patient aspects (patients' experiences, preferences and perspectives) to produce patient-based evidence.

This definition of patient involvement is multidimensional and requires all HTA stakeholders to consider methodologies and approaches that go beyond those traditionally used for evaluating clinical and cost-effectiveness [9].
(In North America, the term patient 'engagement' may be more common than patient 'involvement', but in some languages it is hard to find an appropriate translation for 'engagement', so in this article, we will use the term 'involvement' throughout).

Participation

Considering participation, there is substantial literature about public participation in policy making that dates back to Arnstein's ladder of participation [10]. However, it is important to think about patient participation in HTA, in its specific context. It is not about delegated power or control, which is at the pinnacle of Arnstein's ladder. It is about healthcare systems making difficult decisions on spending priorities.

Looking to publicly funded health research, patient participation has moved from being a tick box on a grant application form, to patients being considered as partners in research [11]. PCORI [12] describes how patients' lived experience and expertise can influence research to be more patient centered, relevant and useful. However, when a contentious decision is to be made that could limit patients access to a health technology, the situation is different. The conflicts of interest that different stakeholders bring must be borne in mind, but this does not prohibit patient participation, it just shapes it.

Gauvin *et al.* [13] provided a framework to consider different types of patient and public participation that could be used in HTA. This was developed further into a mosaic of participation [14] relating to HTA policy, general HTA processes and individual HTAs. This builds on the ideas presented in the OHTAC review [8] that the first step in participation is to understand why you want patients to participate. Facey [14] draws on the HTAi values for patient participation in HTA [15] of relevance, fairness, legitimacy, equity and building capacity and considers who to involve in terms of individual patients, care givers or patient group representatives. The mosaic then offers different mechanisms of participation that will depend on purpose and context, such as providing scientific advice to a technology developer, scoping an HTA or making a decision about a new technology. Examples include public consultation, expert review – answering specific questions, special workshops/meetings with patients or with patients and clinicians, submissions from groups via a prespecified template, participation in multistakeholder meetings as an equal member or invited as a guest expert. Whatever mechanism is used, time should be taken to

evaluate the effectiveness of the approach to ensure that the patient input is really able to influence that stage of the HTA.

Research to produce patient-based evidence

In relation to patient-based evidence, Coulter [16] stated that the HTA research process should include a variety of methods to determine the experience, views and preferences of wide groups of patients.

In recent years, there has been increased interest in the use of patient preference information that arises from economically based research approaches. This research seeks to determine 'the relative desirability or acceptability to patients of specified alternatives or choices among outcomes or other attributes that differ among alternative health interventions' [17]. This form of research is carefully constructed with limited sets of attributes to consider in trade-offs.

Research that arises from the social sciences and humanities is more explorative and reflective. A qualitative research study can evolve as findings emerge; generating insights, rather than producing a definitive result or estimate of effect. It can be undertaken as a standalone study, as a substudy of a clinical trial or virtually, for example, via social media and often does not need many patients to achieve saturation of themes. The challenge is that such research is often not understood by HTA bodies, who equate the word qualitative with descriptive or anecdotal. This could not be further from the truth. The scholarship related to qualitative research is mature, dynamically responsive to changing society and is perfectly suited to asking questions related to patients' use of technologies [18]. At the heart of social science is the careful consideration of what knowledge is, its subjective nature and how evidence should be collected and interpreted. As a result, theoretical frameworks and methodological tools have been developed to ensure rigorous handling of informants' and researchers' subjectivity in qualitative research [18]. Lehoux and Jiminez-Pernett [18] also clearly delineate patients' preferences/perspectives and patient experiences, noting that the former are *"emerging, relational and shifting' whereas patient views are 'the result of context-dependent social interactions wherein perplexing dynamics contribute to the creation of dominant narratives"*.

The lack of understanding in the HTA community about the value of such research is a major limitation. It is essential that we call for more researchers from the social sciences to be employed in HTA bodies to balance the plethora of economists that seem to dominate. We must then all work together to consider how such research can contribute to more rapid HTAs. As the US FDA has produced guidance on submission of patient preference information from manufacturers, can we not encourage HTA bodies to ask health technology developers to submit research into patient aspects that has been undertaken by respected academic bodies? We need to produce guidance on how we will critically evaluate patient-based evidence in our HTA methodology guidelines. There also needs to be collaboration across HTA bodies to produce joint reports that are publicly available, which synthesize existing literature about patient aspects of a particular condition, current use of technologies or experience with new technologies, as was imagined in the eighth domain of the HTA Core Model.

What does patient involvement add to HTA?

I can give examples where patient involvement in a specific HTA has made a difference:

- Patient-based evidence has identified issues about living with the condition and current treatments that are not explained in the clinical description of the disease and identified how patients balance risks, burden of treatment and benefits. These can all impact on the determination of the added value of the new treatment.
- Patient group submissions have included surveys that provide information on outcomes not included in clinical trials such as quality of life data that have been used as utilities in economic modeling or experiences of current treatments that indicate more challenges with a treatment than are apparent in the literature (side effects that limit leaving the house, difficulty in administration of the technology, etc.).
- Individual patients have explained what matters most to them, focusing attention on outcomes that may not be the primary measure in a clinical trial or explaining what a complex outcome means in daily living (ability to dress oneself, shop for food, continue to use a wheelchair and maintain social connections and return to work).

Examples of patient involvement in England and Canada that have impacted HTA recommendations have been reviewed [19], recognizing that patient participation and patient-based evidence can 'put a human face on the evidence' – identifying outcomes that are important to them, addressing gaps in the clinical evidence base, helping

verify or refute assumptions in economic models and informing the deliberative discussions as the evidence from controlled studies is being considered in the local, real-world context.

However, I have also seen many HTAs where patient organizations have spent substantial time and financial resource to make submissions or contribute to an HTA meeting where their input is not listened to or mentioned and clearly do not impact the decision making. This goes against Boivin *et al.*'s premise [20] that to influence decision-making patients must have:

- Credibility (ability to contribute knowledge that is considered valid and relevant and will result in mutual learning);
- Legitimacy (to speak on behalf of others);
- Power and ability to influence.

This is where the context of HTA becomes critically important. If the cost–effectiveness of a new technology is much higher than a standard 'willingness to pay' threshold, it is unlikely that any input can make a difference. On the other hand, do patient groups need to spend resources on contributing to an HTA where the assessment of the clinical and cost-effectiveness is favorable.

Furthermore, patient groups may be asked to contribute to several HTAs within a short timeframe and across different jurisdictions in their area. Given their input is generally not funded by an HTA body, this creates a real opportunity cost for a patient organization, which has a duty to use its funds wisely. So, we need to get smarter in our interactions with patients and patient groups and involve them in ways and at times when their input can influence decision making, generating a dialog to use their unique experiential knowledge. It is time to move the burden of understanding patient aspects from patient organizations back to HTA bodies.

HTA has been described as a bridge between research and decision making. Patient involvement can provide the lights on the bridge. It can alter the value judgments made in an HTA by illuminating areas of unmet need, elucidating the unintended and indirect impacts of the existing or new health technology, identifying outcomes that matter to patients and informing determination of added value. But, patient involvement comes at a cost to all parties involved – particularly patients and their representatives. So we need to rethink how we can invest in patient involvement to make the best use of all forms of research methodologies and collaborate across borders to develop an understanding of patients' perspectives and experiences that may not be health technology specific. We also need to enable patients and their representatives to participate in HTA processes, where they can influence the decision. Finally, we need to encourage all stakeholders to document how they have involved patients in health technology development, assessment and appraisal and reflect on the difference it has made.

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