



Level of Patient Involvement in Health Technology Assessment (HTA) Agencies: A Systematic Literature Review by Alira Health

To characterize the level of current patient involvement in health technology assessment (HTA) in the European, US, and Canadian HTA agencies, Alira Health conducted a systematic literature review of articles published in English between 2016 and 2021 on HTA and patient engagement.

HTAs play an important role in the process of market access for new medicines¹⁻⁴. Structures, methodologies and processes differ from one country to the other, but a common factor is present in all of them: awareness of the importance of including the patient's voice is essential⁵⁻⁸.

Engaging with patients during this process allows them to describe their experience and relevant aspects impacting their quality of life regarding the assessed product, providing the experts with valuable evidence on a patient's perspective^{1,2}. However, while the patient's voice is increasingly being included in the evaluation, patients still perceive that there is a long way to go before their opinion has a real impact on decision-making^{1,3,8}.

REASONS, EXAMPLES AND THE VALUES OF PATIENT ENGAGEMENT

Patient engagement in HTA enhances trust in the decision-making process and contributes to the valuable first-hand experimental knowledge of living with a particular health condition.

REASONS FOR PATIENT PARTICIPATION IN THE HTA^{1,2,4,5}



Transparency, legitimacy, and fairness in decision-making – Patient participation is perceived as a means of enhancing trust in the decision-making process and acceptance of the resulting decisions.



Patients' evidentiary contributions – Patients are seen as contributors of valuable first-hand experiential knowledge of living with a particular health condition. They have experience with the product under assessment, or currently available technologies, the use of associated health services and associated benefits, risks, and side effects.

EXAMPLES OF PATIENT INTERACTION AT THE DIFFERENT STAGES OF THE HTA²



1. Horizon scanning
2. Priority setting or selection of technologies for assessment
3. Conduction of the HTA
4. Review of the evaluation results and generation of recommendations
5. Implementation of the funding recommendations
6. Dissemination of the decisions

It is recognized that patient involvement can and should take place throughout the entire process. In fact, there is evidence that HTA agencies are increasingly engaging patients in all stages.

IMPORTANT VALUES IN THE HTA THAT ARE ADDED BY PATIENT ENGAGEMENT



Patient-based evidence has identified issues about living with the condition and the current treatments that are not explained in the clinical description of the disease, and identified how patients balance risks, burden of treatment, and benefits.



Patient group submissions have included surveys that provide information on the outcomes that are not included in clinical trials. For example, quality of life data have been used as utilities in economic modeling or experiences of the current treatments that indicate there are more challenges with a treatment than are apparent in the literature.



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 their daily life.

PATIENT INCLUSION IN EVALUATIONS

Patients have roles at the different steps of the HTAs in several countries; however, their participation is different during the process and several countries are still barely involving patients^{3,7}.



In Europe, there is an increasing awareness of the benefits of patient engagement in HTA. This is determined at the national and regional levels and is not subject to any European legislation. Consequently, the extent of the patient presence in HTA varies considerably between countries and regions in Europe. NICE in the U.K. and SMC in Scotland offer one of the most formalized and structured European approaches to involvement, where patients participate in meetings, workshops, or committees. In most cases, patients do not have voting rights^{3,7,8}, with some exceptions, including Spain, with the Catalan HTA agency, AQuAS^{9,10}.



In the U.S., patient participation in value assessment has increased and improved considerably since the National Health Council (NHC), launched a Value Initiative in 2016 to support its patient-advocate membership in this regard¹¹.



In Canada, CADTH invites patients to share their perspective in different ways. For instance, in pCODR, which assesses cancer drugs, three patient members with voting rights sit on the Expert Review Committee. For its pioneering role, CADTH has gained international recognition as a leader in the area of patient involvement^{5, 12, 13}.

CHALLENGES FOR PATIENT INVOLVEMENT

Engaging patients in HTA may enrich health policy decisions; however, there are some outstanding questions when engaging patients: methodological, procedural, and normative issues are the most common questions⁴.

METHODOLOGICAL

Which methods are better for eliciting preferences?

How do preferences on an individual level differ from those on a collective level (i.e., preference heterogeneity)?

Which disease-specific (e.g., stage and severity of illness) and sociodemographic patient characteristics affect preferences, and how should we tailor these subgroups?

PROCEDURAL

How can we evaluate the impact of patient preference studies on HTA decision-making?

How should preference studies be evaluated in comparison/addition to clinical and economic evaluation studies?

How can patients be sufficiently trained to perform HTA studies?

NORMATIVE

Do the preferences of representatives of patient organizations/advocacies represent preferences of a broader set of individuals?

Which preferences should be elicited (e.g., patients with or without treatment experience, careers, patient representatives)?

What are patient-relevant outcomes (i.e., health vs. well-being), and should preference studies also focus on process?

Although the level of patient involvement in HTA is increasing, there is still too much heterogeneity between different agencies. The integration of patient preferences is expected to contribute to better decision-making and to increase uptake, adherence, and patient satisfaction. However, there are some methodological and procedural issues that remain unresolved.

ALIRA HEALTH APPROACH

Alira Health helps companies develop 360-degree projects, which enhance and promote patient involvement in the HTA process by designing joint patient engagement and global market access strategies.

Please contact us if you need support for this combined area of expertise and learn more about our Patient Engagement and Market Access services.

References

1. Facey KM. As health technology assessment evolves so must its approach to patient involvement. *J Comp Eff Res.* 2019;8(2):549–54.
2. Scott AM, Wale JL. Patient advocate perspectives on involvement in HTA: An international snapshot. *Res Involv Engagem.* 2017;3(1):1–17.
3. Wale JL, Thomas S, Hamerlijck D, Hollander R. Patients and public are important stakeholders in health technology assessment but the level of involvement is low – a call to action. *Res Involv Engagem.* 2021;7(1):1–11.
4. Huls SPI, Whicchio CL, van Ekel J, Uyl-de Groot CA, de Bekker-Grob EW. What Is Next for Patient Preferences in Health Technology Assessment? A Systematic Review of the Challenges. *Value Heal.* 2019;22(11):1318–28.
5. Berglas S, Jutai L, Mackean G, Weeks L. Patients' perspectives can be integrated in health technology assessments: An exploratory analysis of CADTH common drug review. *Res Involv Engagem.* 2016;2(1):1–13.
6. Wale JL, Sullivan M. Exploration of the visibility of patient input in final recommendation documentation for three health technology assessment bodies. *Int J Technol Assess Health Care.* 2020;36(2):197–203.
7. Stewart J, Clifton E, Macpherson K, Angelova N, Morrison G. Scottish Health Technologies Group: enhancing patient engagement. *Int J Technol Assess Health Care.* 2021;37(e21):1–6.
8. Hunter A, Facey K, Thomas V, Hoery D, Warner K, Klingmann J, et al. EUPATI guidance for patient involvement in medicines research and development: Health technology assessment. *Front Med.* 2018;5(SEP):1–10.
9. Consell Consultiu de Pacients. Departament de Salut [Internet]. [cited 2021 Sep 21]. Available from: https://salutweb.gencat.cat/ca/ambits_actuacio/per_perfiles/Consell_pacients/el_consell_pacients/
10. Àmbit. 2017;
11. Perfetto E, Harris J, Al. E. Emerging good practices fro transforming value assessment: Patients' voices, Patients' values. *Value Heal* 21. 2018:386–93.
12. Mercer RE, Chambers A, Mai H, McDonald V, McMahon C, Chan KKW. Are We Making a Difference? A Qualitative Study of Patient Engagement at the pan-Canadian Oncology Drug Review: Perspectives of Patient Groups. *Value Heal.* 2020;23(9):1157–62.
13. Bidonde J, Vanstone M, Schwartz L, Abelson J. An institutional ethnographic analysis of public and patient engagement activities at a national health technology assessment agency. *Int J Technol Assess Health Care.* 2021;37(e37):1–7.

