



Global Health Technology Assessment (HTA)

Our company works in full accordance with HTA processes in health systems where they have been given a role in delivering transparent, independent evaluations reflecting the full clinical, societal, system-level, and economic value of health technologies. We are committed to collaborating with HTA agencies and other stakeholders to strengthen the design, use, and governance of HTA to improve equitable patient access and healthcare sustainability.

Our company supports the following principles for HTA:

1. HTA value assessments should provide transparent and independent evaluations. Those evaluations should reflect the full breadth of clinical, societal, system-level, and economic impacts of health technologies, independently of affordability concerns.
2. Effective HTA systems should deliver timely, transparent, and actionable recommendations that support patient access to innovation. This requires clear and predictable processes and timelines, multidisciplinary expertise, consistent evidence standards, as well as transparent and fit-for-purpose methods. Health technologies should be evaluated according to standards that were clearly defined at the time that evidence development plans were designed.
3. HTA should be based on the totality of relevant evidence available at the time of assessment. It should include relevant contextual considerations. The process should incorporate broad stakeholder engagement, including patients.

Our Position

Our company is committed to collaborating with HTA agencies and other stakeholders to enable the optimal use, design and governance of HTA that recognizes the value of pharmaceutical innovation, accelerates and expands equitable patient access, and promotes the sustainability of healthcare systems. Our company supports the following principles for HTA and other value frameworks:

- 1. HTA value assessments should provide transparent and independent evaluations that reflect the full breadth of clinical, societal, system-level, and economic impacts of health technologies, independently of affordability concerns.**

HTA should enable efficient resource allocation to prioritize technologies that provide optimal value and benefits for patients and healthcare systems. It is important to have transparent methods to conduct holistic value assessments.

- The full value of health technologies includes:
 - Direct medical effects to the patient (e.g., health benefits on disease and quality of life, adverse events, hospitalization rates)
 - Impact on caregivers and families (e.g., reducing caregiver burden, improving patient independence and autonomy) and society (e.g., improving productivity by reducing absenteeism, improving equity through addressing disparities in access to healthcare)
 - Impact on healthcare systems (e.g., improving health care efficiency, shifting service delivery from in-patient to out-patient settings, addressing non-medical effects, creating supply chain resilience, etc.)
 - Economic and fiscal impacts (e.g., generating savings beyond the health system budget)
 - Value and affordability are distinct but related considerations. We recognize that affordability is a central concern for healthcare systems, and we support the use of tools like innovative financing models and value-based/risk-sharing agreements to manage short-term impact without compromising long-term value.
 - Ex post adjustments to evidence plans to meet new or revised standards for HTA evaluation introduce risks of bias and may negatively impact access for patients.
 - To ensure independence, the individuals or organizations conducting the value assessments should be separate from those making decisions related to clinical practices, budgeting, pricing, and reimbursement.
 - Any collaboration and joint work between HTA agencies should focus on the common aspects of the countries and regions involved. Key elements of HTA analyses vary across healthcare systems, including current treatment options, patient population, cultural norms and values, healthcare costs, individual willingness to pay, and disease burden. The assessment of economic value should remain at the country level since affordability, resource-allocation, and other economic questions are country-specific and cannot be transferred across different healthcare systems.
- 2. Effective HTA systems should deliver timely, transparent, and actionable recommendations that support patient access to innovation. This requires clear and predictable process and timelines, multidisciplinary expertise, consistent evidence standards, as well as transparent and fit-for-purpose methods. Health technologies should be evaluated according to standards that were clearly defined at the time that evidence development plans were designed.**

For HTA systems to be effective, they should:

- Align the HTA process within its local mandate, guidelines, and priorities. This involves considering key factors like disease burden, treatment effectiveness, practice variations, and resource allocation. HTA should prioritize timely patient access to innovation, cost-effective population health recommendations, evidence-based decision-making, and transparent processes as guided by the host country.
- Ensure governance and processes are transparent and relevant. To maintain trust and relevance, HTA processes should be transparent about their methodology, structures, timelines, and decision criteria. Agencies should also regularly review and update their findings considering substantial new evidence.
- Acknowledge data and methodological limitations, recognizing all sources of potential bias and uncertainty. Sufficient sensitivity analyses should be conducted to understand any uncertainty in the evidence that may be important to the decision.
- Ensure adequate expertise and infrastructure. Where HTA processes are in place, the system should ensure there is adequate expertise to appropriately interpret the HTA to inform decision making.

3. HTA should be based on the totality of relevant evidence available at the time of assessment, including relevant contextual considerations. The process should incorporate broad stakeholder engagement.

- HTA should consider data from all relevant sources including real-world data (RWD) and evidence (RWE), observational research and patient generated data. Data ranking should be based on study quality, with clear disclosure of assessment standards and not be limited to peer-reviewed publications or publicly available data. Consensus approaches to handle uncertainty in these data are crucial for effective HTA utilization.
- The evaluation should consider relevant context – for example, maturity and certainty of evidence should be weighed against factors such as high unmet medical need.
- HTA should include healthcare providers, patients, citizens, technology developers, payers and government decision makers, and other groups if appropriate. The unique needs for each stakeholder, especially patients, can help define perspective on a condition in the value assessment. To ensure these stakeholders can effectively contribute to the HTA process, agencies should consider the time and training needs of each stakeholder.^{1,2}

¹Arntsen, Kathleen A., Linda G. Blount, Bradley J. Dickerson, Catherine P. Koola, Yvette Venable, and Patrick Wildman. 2022. "Patient-Centered Health Technology Assessment: A Perspective on Engagement in Health Technology Assessment by Three Patient Organizations and a Health Technology Assessment Body." *International Journal of Technology Assessment in Health Care* 38 (1). <https://doi.org/10.1017/s0266462322000587>.

²Bertelsen, Neil, Lode Dewulf, Silvia Ferrè, Rebecca Vermeulen, Karlin Schroeder, Laureline Gatellier, Ify Sargeant, Daniela Luzuriaga, Hayley Chapman, and Nicholas Brooke. 2023. "Patient Engagement and Patient Experience Data in Regulatory Review and Health Technology

Background

The goal of HTA is to inform healthcare decision-making and improve access to and utilization of technologies that improve healthcare outcomes effectively and efficiently.¹ Therefore, HTA should provide a comprehensive analysis that considers the medical or clinical benefits of the technology, as well as the ethical, economic, and societal factors. Such factors might include improved patient health outcomes, quality of life, treatment experience and adherence, and ability to return to work.

Globally HTA policies and methodologies have been rapidly evolving. National HTA agencies are seeking to foster greater collaboration such as the European Union's HTA Regulation and the cross-border alliance of HTA including Australia, Canada, the United Kingdom, Ireland, and New Zealand, among others. As collaboration and cooperation grow, the evolution of HTA should be guided by alignment on shared principles, rooted in evidence-based practices and established methodologies, while ensuring accessible, transparent dialogue among multi-sectoral stakeholders including clinicians, patients, regulators, payers, HTA agencies, and developers of medicines and technology.

Collaboration must explicitly recognize and uphold the distinct mandates and evidentiary requirements of national and local HTA agencies.

Definitions

HTA: Health technology Assessment (HTA) is a multidisciplinary process that applies scientifically based methods to assess the value of a health technology across different stages of its life cycle.³

Assessment: A Global Landscape Review." *Therapeutic Innovation & Regulatory Science* 58 (1): 63–78. <https://doi.org/10.1007/s43441-023-00573-7>.

³O'Rourke, Brian, Wija Oortwijn, and Tara Schuller. "The New Definition of Health Technology Assessment: A Milestone in International Collaboration." *International Journal of Technology Assessment in Health Care* 36, no. 3 (2020): 187–90. <https://doi.org/10.1017/s0266462320000215>.