**ISSUE:** Healthcare decision makers worldwide are seeking to improve health outcomes across their populations. Innovation plays a critical role in this as new and improved healthcare technologies leverage scientific and engineering advancements for better patient and public health. As these new technologies become available, policymakers are increasingly employing health technology assessment programs (HTAs) to help inform their decisions on coverage, reimbursement and utilization. HTA is defined as a “multidisciplinary field of policy analysis, studying the medical, economic, social and ethical implications of the development, diffusion and use of technology”, which is a “dynamic, rapidly evolving process, embracing different types of assessments that inform real-world decisions about the value [i.e. benefits, risks, and costs] of new technologies, interventions, and practices.”

Properly designed HTAs that focus on the best outcome for patients and society - based on robust data and proven methodologies - are an important tool for governments/payers when they are deciding on the deployment of new technologies and the allocation of healthcare funding. HTAs that are poorly designed, however, can have an adverse impact leading to decision-making that is not in the best interest of patients or society. For instance, a poorly designed HTA program can result in unfair or unjustified market access limitations that may negatively impact patient outcomes and discourage further industry investment in innovation for that country.

**POSITION:** When HTAs are used to inform healthcare decision making by governments, payers and healthcare providers, their design and recommendations should be based on the impact of a healthcare technology on patient care, disease severity, societal impact, and community values. They should be administered in an efficient manner that promotes patient access to high-quality healthcare and be developed with transparency and in consultation with a broad range of stakeholders.

The following are key elements for best practice in HTA programs:

1. **The primary objective of an HTA program should be to improve health outcomes for patients and society by balancing clinical effectiveness (also called clinical utility) for in vitro diagnostics, societal values, and health system impact through medical technology assessments.**

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1 Health care technology within the concept of HTA is defined as consisting of: drugs, biologics, some classes of devices, medical and surgical procedures, support systems, and organizational, delivery, and managerial systems.

2 Drummond MF, Schwartz JS, Jonsson B et al. Key principles for the improved conduct of health technology assessments for resource allocation decisions. *Int J Technol Assess Health Care* 2008; 24(3):244-258.


2. In evaluating the impact of a medical technology, HTAs should prioritize the clinical and health outcomes of a technology on both the patient and the population at-large over other considerations.
   - HTA should focus first on clinical and health care outcomes, and may secondarily consider health economic analyses to inform the cost impact with respect to the value delivered by the technology.

3. If an HTA considers cost, the assessment should utilize an appropriate analytic approach rather than viewing costs in budget silos or solely by acquisition cost.
   - An appropriate analytic approach, timeframe and perspective should be selected for the health economic analyses.
     - For instance, health economic analyses may include economic evaluation methods suitable for long-term value assessment (such as cost-effectiveness, cost utility or cost-benefit analysis) and/or budget impact analysis suitable for affordability and budget planning considerations.
     - The method of analysis and measure of benefit needs to be flexible to account for the varying characteristics of different technologies.
     - To accurately reflect value, economic analyses should at least include medical and economic outcomes impact across the health system, work flow efficiencies, a technology’s impact in preventing future costly complications (such as infections, long term disease co-morbidities, follow-up procedures, etc.), as well as patient health outcomes.

4. The HTA methodologies employed for medical devices and diagnostics should reflect the attributes of the device and in vitro diagnostic development and innovation process.
   - Medical devices, including diagnostics, are different from pharmaceuticals. Utilizing a pharmaceutical model for health technology assessment is often not appropriate for devices.
     - For example, the health impact of diagnostic testing depends on numerous variables including the outcomes of subsequent clinical interventions applied following a diagnostic result.¹

5. HTA programs should prioritize assessments for technologies that are likely to have high impact on population health and are relevant to the health priorities of the government and payers.
   - Program managers should conduct realistic evaluations to determine which technologies should be prioritized for HTA analyses. These evaluations should be based on whether the technology addresses an unmet healthcare need and/or a priority of the government. Another relevant factor for prioritizing a technology for assessment may be to help accelerate its rapid dissemination.
   - Governments should ensure that resources are available to effectively conduct priority HTA efforts and exclude or defer unnecessary assessments to avoid delaying high-priority product reviews.

6. **Programs should be administratively efficient and timely.**
   - HTA programs should be designed and executed in an administratively efficient manner to avoid delays in patient access to new technologies.
     - To streamline the HTA process, the roles and responsibilities of government and non-government entities involved in HTAs should be clearly defined to avoid unnecessary and poorly-designed processes.
     - Responsible entities should be held accountable to deadlines and performance measures.
     - Programs should scan the medical technology landscape for emerging technologies to allow them to be technically and administratively prepared to conduct future HTAs in an efficient manner.

7. **HTAs should be focused on well-defined questions, based on robust and relevant evidence, and employ strategies to avoid delays to patient access.**
   - Research questions being addressed should be defined and allow for stakeholder input prior to finalization.\(^vi\)
   - Uncertainties and gaps in recommendations should be acknowledged and explained.
   - Information gaps that could prevent or delay assessments, should be addressed in a consultative manner and mitigated when possible.
     - Some approaches to avoid delays include conditional recommendations, coverage linked to further evidence development, post-market monitoring, and cost-sharing with industry while evaluations are being finalized.
     - Assessments should be reviewed and revised periodically as new information becomes available.

8. **Processes should be consultative and transparent.**
   - **Consultative:**
     - Participation and input from a diverse group of key stakeholders, including patients, key opinion leaders, medical societies, and industry should be incorporated in a structured manner.
     - Assessments should incorporate societal values including perspectives on access and innovation.
     - Initial decision statements, evidence under review and draft recommendations should be posted for public comment, and these comments should be considered in the development of interim or final recommendations.
   - **Transparent:**
     - Processes should be publicly communicated and clearly explained.
     - Applicants should be kept informed of progress throughout the HTA process.
     - Reviewers should be independent and potential conflicts of interest publicly disclosed.
     - Recommendations should be clearly communicated along with supporting facts and rationale and the conditions upon which recommendations are predicated.

\(^vi\) Typically, HTAs analyze if a technology:
1. is clinically effective
2. is safe
3. offers improved value
4. is comparable to other technologies
• Final HTA recommendations should have a clearly defined mechanism for handling appeals from stakeholders.
  o For instance, a clear process should be outlined to address incorrect information and to re-open an assessment when new information becomes available.

9. **HTAs should not be used or perceived as an additional hurdle for approval from regulatory agencies.**

• Regulatory agencies have a distinct role in reviewing products for safety, efficacy/accuracy and quality.
  o The role of HTA is not to limit product availability, but to selectively assess important technologies to guide their use and support a system of incentives to drive implementation of best healthcare practices.
• The majority of products approved by regulatory agencies are not candidates for HTA. Therefore, HTA principles should not be used to delay regulatory market approval as this could negatively impact patient access to many technologies.

Background:

**Overview**

Health technology assessment (HTA) is a multidisciplinary approach to evaluating the medical, social, ethical, and economic implications of the development, diffusion, and use of health technologies.¹ They can apply to a broad range of health technologies, including drugs, biologics, devices (including diagnostics), medical and surgical procedures, and support systems (such as electronic payment record systems and drug formularies).² The purpose of HTA programs is to provide policymakers, payers, health professionals, and health consumers with information to understand the benefits and comparative value of health technologies and procedures. Well-designed HTA programs and analysis can help:

• Expedite patient access to cost-effective health technologies that improve health outcomes
• Minimize the utilization of technologies that are less effective; and
• Inform private and public sector investment in healthcare innovation, in light of limited healthcare resources.

However, poorly-designed systems for overseeing and conducting HTAs can have an adverse impact on patients and health systems, including:

• Delayed access to effective healthcare interventions
• Missed opportunities to avoid future costs of advanced illnesses through early diagnosis and prompt initiation of treatment
• Misinformed or ambiguous recommendations to healthcare providers as a result of inappropriate methodology and/or inadequate consultation
• High administrative burden and cost on public and private stakeholders that divert scarce resources away from high-impact activities to low-impact activities.

**HTA Design**

The primary goal of HTA programs should be to improve patient and health system outcomes, in the context of the technology’s role in advancing a country’s healthcare goals over time. While near-term budgetary constraints are inevitably one of many considerations, they should not be a prevailing factor in HTAs. Further, HTAs should not be used or perceived to be used to delay patient access to high-quality technologies.
When reviewing a technology, an HTA should incorporate a range of inputs relevant to the technologies being evaluated. To do this effectively, programs should actively solicit input from key stakeholders, including patients and subject matter experts from the medical, scientific and industrial sectors. This is important to ensure that assessments are informed by the best available expertise and reflect the values of the communities in which the outcomes of an HTA will be deployed.

Assessments should also reflect the value of a technology in enhancing disease detection, prevention and management (including patient compliance) and their impact on avoiding future costs. Key considerations such as the following should be included in study designs:

- Many diseases can be treated more effectively and at a lower cost when they are diagnosed early and treated accurately and appropriately.
- Early diagnosis can allow for effective interventions, whether therapeutic or lifestyle, and avoid more serious morbidity and complications that arise from advanced disease states.
- Patient compliance with therapy is essential to ensure that patients benefit from them and that health resources are used efficiently.

In developing study protocols, HTA agencies should always keep in mind that the research and development, regulatory review, production and clinical application of medical devices and diagnostics are distinct from those of pharmaceuticals. One example of this distinction is in the determination of the clinical efficacy of a diagnostic device. To make this determination appropriately, an analysis must incorporate the analytic validity, clinical validity and clinical utility of an in vitro diagnostic test. These key factors determine the ability of a laboratory to run a test and the ability of a clinician to meaningfully interpret a result to inform a diagnosis or treatment strategy. Considerations like these are unique for diagnostics tests and would not be present in an HTA process designed for a pharmaceutical.

Also, medical devices and diagnostic tests are characterized by a high rate of innovation, often undergoing continuous and iterative improvement, with shorter product lifecycles and investment recovery periods compared to pharmaceuticals. This means that the evidence connecting a particular device to improved long-term outcomes (which takes years to study) may be out of date and/or irrelevant by the time an HTA is complete since new iterations of the technology may, by then, be the most common product of its type used in the marketplace. HTAs for devices and diagnostic technologies should be designed to reflect these attributes and limitations to the evidence base.

Health technology assessments can take different forms depending on the goals of the analysis and the characteristics of the technology and the population to benefit from it. Consequently, numerous health economic analytical approaches have been developed for HTAs. The suitability of a particular approach depends upon the availability of data and other resources.

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vii The term “community values” reflects the interest in involving citizens in policy development to ensure that decisions incorporate broad social values of the public. A common method to elicit ethical and social values and ensure HTA decisions reflect community values is to hold a structured discussion forum or “Citizen’s Panel.” Creating a forum for feedback and ensuring participation of community members can assist in resource allocation and confidence that the assessment decision reflects the many perspectives. It is essential that these forums are conducted with established methods for eliciting deliberations and that biases are minimized in the panel selection process.
In general, HTA programs utilize one of several economic evaluation methods or use budget impact analyses. Common economic evaluation methods include cost-effectiveness analysis, cost-utility analysis and cost-benefit analysis, all of which generally focus on long-term economic impact. In contrast, budget impact analysis is typically concerned with costs over a shorter time horizon. Where both an economic evaluation and a budget impact analysis are conducted as part of an HTA, they are expected to be driven by the same core assumptions and evidence and should utilize data that is consistent for economic evaluation and budget impact analysis.

When designing a device or diagnostic HTA, the analysis should be focused on questions that are clearly defined and supported by robust and relevant evidence. There are unique differences between various device and diagnostic technologies that require a customized approach to HTA research. Poorly defined questions or equivocal or inaccurate data can lead to recommendations that are not in the best interest of patients or public health or in conclusions that are impractical for application.

During the course of conducting an HTA, gaps in information may be identified that can prevent or delay a timely and accurate assessment, which in turn can delay patient access to important technologies. When this occurs, the organization conducting an HTA should consult with stakeholders - including manufacturers - in order to identify strategies that can lead to sound solutions. These solutions may include conditional recommendations, post-market monitoring, and cost-sharing with industry to develop additional data. Also, because available information on particular health technologies may change over time, assessments should be revised periodically. This is important to ensure that they remain relevant and focused on the best outcomes for patients.

*Stakeholder Consultation and Transparency*

In all of these activities, transparency, consultation, and timeliness are essential to ensure that the outcomes of HTAs are well-informed, clear in scope and strength, and are regarded as non-biased. Participation and input from key stakeholders, including expert input from patients, key opinion leaders, and industry should be incorporated in a structured manner. Processes, including criteria and information needs, should be publicly communicated and clearly explained. Applicants and other stakeholders should be kept informed of progress throughout the HTA process.

When complete, the outcomes of an assessment should be clearly communicated to stakeholders along with supporting facts, rationale and any limitations of the recommendation. This is important so that all stakeholders have a clear understanding of both the scope and strength of recommendations.

*Program Effectiveness and Efficiency*

In addition to the design and execution of individual assessments, the programs that conduct HTAs should be designed in an administratively efficient manner and not serve as a disincentive to investments in innovation. For example, not all technologies warrant an assessment, and program managers should evaluate which technologies to assess so that resources devoted to HTAs can be directed toward high-impact priorities. Unnecessary assessments divert government and payer resources from high-priority initiatives, delay the availability of quality products to patients, and have low-impact in determining the efficient allocation of health resources.

A clear definition of the role of different entities involved in HTA programs and coordination of those entities is another critical aspect of efficient administration. Lack of clarity and coordination of roles can result in duplicative and poorly-designed processes that adversely impact the quality of assessments and waste resources from both public and private sector partners. Program performance measures should
also be established and responsible entities held accountable to those measures. Additionally, in order for programs to be prepared to conduct future assessments, strategies to maintain a trained workforce, identify emerging technologies, and identify expert input should be employed. This allows programs to identify gaps in expertise and resources so that assessments on new technologies are not unnecessarily delayed.

Conclusion

HTAs can be an important tool for leveraging scientific and engineering advancements for better patient and public health outcomes. When appropriately executed, they can bring rigor to decision-making on coverage and reimbursement and can help accelerate the dissemination of evidence to guide technology adoption.

However, poorly executed HTA programs can have a counter productive effect on patient care and health systems if they become a tool that reduces access to medical technology or become a disincentive to investment in healthcare innovation.

Applying the best practice elements presented in this paper will help to ensure that HTA programs contribute to better patient and public health and support evidence-based decision making by policy makers, payers, and healthcare providers.
Appendix

Types of Health Economic Analyses

HTAs employ two general types of review: economic evaluation and budget impact analysis (BIA).

- Economic evaluation analysis addresses the additional health benefit gained from investment in a technology, such as the cost per additional quality adjusted life year (QALY) gained, by comparison of that technology to a different intervention or to no intervention.
- Budget Impact Analysis (BIA) addresses the affordability of the technology, such as the net annual financial cost of adopting the technology for a finite number of years.

Although BIA and an economic evaluation have many similar data and methodological requirements, there are key distinctions between the two approaches:

- BIA is not an economic analysis, but is based on the principles of accounting
- Economic evaluations are typically not modeled for the actual anticipated size of the patient population, whereas this is required for BIA
- Economic evaluations report costs and consequences (health outcomes), while BIA reports costs only
- The results of economic evaluation are presented as the discounted present value of costs and effects in one period, while BIA report the costs for each year in which they occur

Economic Evaluation
Analytical methods for economic evaluation include:

- Cost-effectiveness analysis (CEA): a comparison of costs in monetary units with outcomes in quantitative non-monetary units, e.g. reduced mortality or morbidity
- Cost-utility analysis (CUA): a form of cost-effectiveness analysis that compares costs in monetary units with outcomes in terms of their utility, usually to the patient, measured, e.g., in QALYs
- Cost-benefit analysis (CBA): compares costs and benefits, both of which are quantified in common monetary units.

Budget Impact Analysis
Budget impact analysis (BIA) is a tool to predict the potential financial impact of the adoption and diffusion of a new technology into a healthcare system relative to available financial resources. Although different specifications may be used for a BIA, it generally refers to an analysis of the added financial impact of a new health technology over a finite time period.

Comparison of Health Economic Analysis Types

<table>
<thead>
<tr>
<th>Parameter</th>
<th>Economic Evaluation</th>
<th>Budget Impact Analysis</th>
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<tbody>
<tr>
<td>Purpose</td>
<td>Efficiency of alternative technologies</td>
<td>Financial impact of introducing a technology</td>
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<tr>
<td>Study Timeframe</td>
<td>Usually long-term (e.g., lifetime)</td>
<td>Usually short-term (1-5 yrs.)</td>
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<tr>
<td>Health Outcomes</td>
<td>QALYs (quality adjusted life years), life years or other outcome</td>
<td>Excluded</td>
</tr>
<tr>
<td>Results</td>
<td>Incremental cost per unit of health outcomes achieved</td>
<td>Total and Incremental Annual Costs</td>
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1 International Network of Agencies for Health Technology Assessment 2002. Available at http://www.inahta.net/
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