

Health Technology Assessment (HTA): Good Practices & Principles

FIFARMA, 2016



Key Points for Decision Makers

- *Health Technology Assessment (HTA) refers to the scientific-multidisciplinary field that addresses in a transparent and systematic way the clinical, economic, organizational, social, legal, and ethical impacts of a health technology, considering its specific healthcare context.¹*
- *In both developed and emerging markets, HTA is frequently used by governments and payers as a way to allocate limited health care resources and are intended to maximize resource impact for the health needs of the population.*
- *However, if done improperly, the HTA process can undermine access for patients to the appropriate technology, inefficiently allocate resources, and diminish physician freedom to prescribe and patient choice.*
- *Therefore, the following principles must be followed when considering HTA: transparency, independency, fairness, consultation, and predictability. **It is still the view of FIFARMA that HTA has to be value-oriented.***

¹ Definition of HTA by HTAi.
<http://www.htai.org/htai/what-is-ha.html>

I. Government's cost-containment measures: current status & issues

Improved economic conditions and advances in health care have led to both increased lifespans and workforce productivity around the world. While dramatically improving patient well-being, these developments have led to a demographic shift towards ageing populations and accordingly an increase in spending on patient care.

In response to these economic and societal challenges, governments are increasingly relying on budget and price-setting mechanisms to contain health care costs. However, in an attempt to decrease short-term health expenditures, cost-containment measures frequently undervalue innovation, may adversely affect patient access to optimal health care, and may lead to increased spending in the long term.

Conversely, and not unlike preventative care for individuals, an early investment in health care may result in increased future health benefits and decreased downstream costs.

Optimization focused on health outcomes, instead of cost-containment measures, should focus on patient needs first and the long-term sustainability of health care systems rather than short-term cost cutting.

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Otherwise, any cost containment measure would lead to a negative impact in the economic, humanistic and clinical outcomes.²

II. FIFARMA's position on government's cost-containment measures.

Healthcare systems would need to remove cost containment measures (price restrictions and utilization controls) that could impair access for patients and/or stifle innovation.

We recognize that healthcare systems need to control and manage healthcare costs, however we would argue that this could be achieved by using existing resources more efficiently (in all the health care interventions, instead on only on technology) and enabling other sources of funding.

Long-term sustainability of the health care system depends on collaborative, transparent government interaction with stakeholders to achieve common goals and reward innovation through fair pricing and constructive reimbursement environments.

² Pollard, S et al. *The human cost of pharmaceutical price controls in Europe*. 2004.
<http://cmp.roularta.be/cmdata/Attachments/site4/2004/30/HumanCost.pdf>

III. Principles regarding HTA.

FIFARMA recognizes the value of HTAs and recommends the following key general and specific principles to which governments and payers should adhere as they evaluate the substance of assessments or consider designing an HTA system.

General Principles:

HTA's processes (assessment and appraisal) must be:

1. Transparent and Independent in both process and outcome
2. Consultative and participative (multi-stakeholder, industry included)
3. Fair, credible and unbiased scientifically-based review
4. Predictable and stable, with optimum and defined timelines
5. Value-oriented

Specific Principles:

1. Transparency:
 - Explicit documentation of:
 - The HTA process to be followed
 - The methods to be implemented, considering MCDA (Multi-Criteria Decision Analysis).
 - The decision criteria and weighting
 - What is expected from manufacturers

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2. Consultative

- Early notification of manufacturers when one of their products is targeted for HTA
- Involvement of stakeholders, including manufacturers, in setting the scope of an HTA (defined scope)
- Patient-centered; more participation of patients and their associations in the HTA process
- Consideration of all stakeholder comments, with explicit responses

3. Fair, credible and unbiased scientifically based review

- Detailed technical report of the HTA, including availability of any model used
- Protection of confidential information and agreements.
- Separated HTA, regulatory and procurement bodies (assessment and appraisal).
- Clear appeals process with the ability to impact the final decision.

4. Predictable and stable, with optimum and defined timelines.

- Opportunity for review (with sufficient time) and commenting

5. Value-oriented.

- Innovation rewarded.

- New methods (MCDA: Multi-Criteria Decision Analysis)

IV. Aspects of interest for FIFARMA's position on HTA.

FIFARMA's position is focused on 5 aspects of interest, as follows:

1. Selection and prioritization of medical technologies to be assessed.
2. Objective and scope of assessment
3. Methodology of assessment
4. HTA process (with emphasis in timing, transparency, consultative opening and independence)
5. Quality Assurance of HTA

V. Selection and Prioritization of medical technologies to be assessed.

- The right to present a request for health technology assessment must be guaranteed to all the stakeholders
- In case technologies to be assessed are pre-defined by an institution (e.g., Ministry of Health, HTA body, other), the following is expected:

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- There must be a clear criteria and process to select medical technologies to be assessed by the HTA body.
- The criteria to be used must be published publically (transparence) with enough time for manufacturers to prepare a robust dossier.
- The right to present a request for assessment must be guarantee to all relevant stakeholders
- A comprehensive list with technologies to be assessed must be published in a public way at least 3-6 months before starting the evaluation and during this time has be open to receive society feedback
- Any decision about technologies to be evaluated has to be discussed and published in order to demonstrate inclusion of all the relevant users (from patients to policy makers) and transparency in the process

VI. Objective and scope of assessment

- The current scope of assessment in Latin America can be summarized in two topics:
 - Inclusion to an institutional list or inclusion to a National list of services
 - Cost-containment and restriction-prescription policies to consider a product as priority to be purchased by a national health care
- In this point the issue is that, the analysis is considered under a perspective of cost-containment and prescription-restriction policy to leverage savings by their respective health care budgets. Cost-containment is supported by cost-effectiveness analysis under an arbitrary threshold³. Prescription restriction policies are supported by budget impact analysis with the main consideration of a restricted financial resource.
- The primary goal of an HTA should be to optimize and accelerate patient access to important therapies, not minimize cost at the

³ http://www.who.int/health-technology-assessment/HTA_November_meeting_report_Final.pdf

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expense of patient health. Wherever possible, the HTA should be tailored to the individual characteristics of a specific health care system, and results should not be assumed readily transferable from one system to another.

- HTAs should take into consideration a range of factors to ensure customization to the population and health care system, such as: local demographics and epidemiology, costs, disease burden, standards of care and clinical practice, , health care investment, agenda priorities, and payer and societal, including patients, choices around funding. Social, ethical and legal aspects have to be also considered (eg EUnetHTA core model⁴). Additionally, the outcomes of HTAs should be aligned with available GDP investment on health.
- Ultimately, HTAs should be used not just for evaluate the economic efficiency of treatments but, together with other criteria, to define future budget allocations and to improve health care quality and efficiency across the entire health care system.

- Having into account that the current scope of HTA is based mainly in financial concerns in their health care budgets to try to preserve sustainability in them more than improving and measure health outcomes in the population, the

FIFARMA position is:

- Any technology analysis has first to demonstrate a benefit in terms of clinical and humanistic outcomes to the final consumer (in more frequency are patients)
- Once is demonstrated a clinical and humanistic benefit, these results have to be discussed by people are representing society and the health condition in analysis, from the point of view of ethical and legal considerations
- After the technology has been accepted as relevant and useful demonstrating the benefits mentioned before, the final analysis has to demonstrate an improvement in the burden of disease in terms of final user welfare, use of resources and budget impact considering all the costs are involved by a holistic perspective and not just only the technology acquisition

⁴ <http://www.eunetha.eu/hta-core-model>

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cost (all the costs related to prevention, diagnosis, care, treatment and follow up of the health condition in mention and analysis)

years and Latin America is clearly a late adopter of this, when even in UK has been criticized due to the restriction policy (with financial arguments predominantly).

VII. Methodology of assessment.

- In general, the most accepted methodology in the formal HTA bodies that are currently working in Latin America is based on the cost – utility analyses using Quality – Adjusted Life Year (QALY) as main outcome to measure and calculate Incremental Cost Effectiveness Ratio (ICER) in order to get willingness to pay with restrictive thresholds⁵
- This methodology has been used broadly by NICE in UK in the last 15

- The decisions must not be taken with the only intention to meet health care budgets, because it can generate bias to prefer or select some technologies over others.

- **FIFARMA's position about the methodology of the assessment** is as follows:

- Needs to be clear, timely and transparent.
- It should involve all stakeholders (manufacturers, health care providers, payers and patients)
- Comparators: Should be relevant to clinical practice and appropriate. It should not only involve the drug, but a broader concept around the treatment
- HTA should approach new technologies applying not only cost effectiveness approach, but it should be more inclusive of different methods such as CER

⁵ WHO, November 2015.

USING HEALTH TECHNOLOGY ASSESSMENT FOR UNIVERSAL HEALTH COVERAGE AND REIMBURSEMENT SYSTEMS, pags 9-10:

"GDP based thresholds arose from the Commission on Macroeconomics and Health report, presented to the WHO Director General in 2001, and were adopted by WHO-CHOICE, for use in normative, global cost-effectiveness analysis. These global level cost-effectiveness studies are intended to indicate interventions which a country may consider, but are not intended to be prescriptive lists of interventions which should be funded. Subsequently many countries have developed CETs which are based on GDP, which is considered to be a misuse of this form of threshold setting. Using cost-effectiveness information in decision-making remains challenging. The view of the WHO Secretariat is that fixed cost-effectiveness thresholds should not be used as an isolated criterion for decision-making. Above all, countries should generally not use '3 x GDP per capita' for national funding decisions or for setting the price of interventions, including new pharmaceutical products. WHO-CHOICE has never recommended this practice at the country level for budget decisions, which is a distortion of the intention and meaning of the thresholds proposed by the Commission on Macroeconomics and Health. Fixed thresholds in and of themselves are not very informative, except perhaps in narrowing the field of options for consideration when used in conjunction with other criteria".

http://www.who.int/health-technology-assessment/HTA_November_meeting_report_Final.pdf

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(Comparative Effectiveness Research) and MCDA⁶

- MCDA must have special development and use during the assessment, because costs and efficiency are not the only criteria for driving a final recommendation about a technology. FIFARMA has a specific position paper on MCDA due to the importance and also opportunities of implementation in Latin America.
- Ethical considerations must be part of both the used criteria and the analysis process
- Societal perspective should be incorporated and adequate Latin America data sources should be developed to reduce analysis uncertainty
- Budget impact analysis should be relevant to all

stakeholders and not only focused on drug costs

- Literature review should access all relevant databases and disclose data limitations on the analysis.
- In order to ensure a fair and credible HTA decision, HTA bodies must provide clear and transparent rationales, requirements, and procedures for assessment. It is important that not only the HTA process itself, but also the results of the assessment, are transparent and objective

VIII. HTA process (with emphasis in timing, transparency, consultation and independence)

- In general, the assessment processes nowadays in Latin America are delayed. There are not clear public processes through the methodology, until the appraisal or recommendations. They are usually perceived with the clear intention to support price reductions, cost-containment and restriction-prescription policies in order to leverage the budget impact and alleviate financial issues.

⁶ Regarding MCDA, please see FIFARMA's position paper: **"Utilization of Multiple-Criteria Decision Analysis (MCDA) to Support Healthcare Decision Making"**.

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- In order to assure that a health technology assessment is fair, transparent, inclusive, independent and on-time, it must fit some specific characteristics from the FIFARMA's standpoint:

FIFARMA's position on Timing:

- For the purposes of marketing authorization, the HTA should be separated from the regulatory review and based on objective, scientifically verifiable criteria including efficacy, safety, quality, and, when possible, patient health outcomes. Moreover, in instances when HTAs are delivering guidance for formulary or market inclusion, the evaluating body should be independent of the budget holder. The HTA should be timed to conclude immediately following marketing authorization, and should aim to minimize delays in patient access to innovative technologies.
- An HTA should be performed soon after regulatory approval, when all the relevant information for optimal HTA is available, while providing timely access to the market during the assessment. In any event, HTA bodies should notify pharmaceutical companies about an intended assessment at an early stage. For

products under clinical development, the notification should occur as close to the beginning at phase II-b, as possible, which allows for the collection of outcomes relevant for the assessment during the development and registration phase of the drug. In addition, the HTA body should be available for consultation by the manufacturer as early as possible in the development and registration phase: dialogue prior to the HTA assessment would be crucial, especially in emphasizing where more holistic decision-making is needed and in preparing the way for utilization, budget impact and affordability discussions

- Assessments that directly affect funding decisions should be conducted in a timely fashion, ideally lasting no more than 6 months. If immediate access is not granted after regulatory approval and before an HTA is conducted, conditional reimbursement connected to the collection of real world data is an acceptable alternative.
- HTA bodies should engage in a continuous dialogue with manufacturers throughout the entire assessment process. Any discussions about sensitive data (pricing, for example) between the

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manufacturer and the HTA body should be conducted confidentially to honor and secure current and future agreements.

- If assessments are conducted for mature products, a dialogue should begin no later than six months prior to the assessment. This allows time for the manufacturer to prepare the appropriate materials and data required. This pre-assessment period should not be less than the time allocated by the HTA body for the actual assessment.

FIFARMA's position on Transparency:

- HTAs should have a formal basis or law establishing how they are to be used, including explicit direction on purpose, scope, influence, and methods. The HTA process must be transparent and open to the public, including patients. HTA organizations should make publicly available a list of health technologies under review and should notify manufacturers of assessments for both pre- and post-approval products. Stakeholders (e.g., payers, patients, manufacturers, and clinicians) should be allowed interim dialogue and ample opportunities to comment before guidance or assessments are finalized to ensure valid and reliable results and

recommendations. There must be an appealing process for submitting new and valid information that may change a previously reported HTA result. Finally, reviews should be conducted according to clear guidelines and a predictable timeline.

- Methodology and outcome parameters for use in the HTA must be communicated in a timely manner, and clear timelines established for the performance of the assessment. The general guidelines for the performance of these assessments should be published and updated periodically (and these guidelines and updates should be subject to administrative notice and comment policies). In addition, models used by the HTA body must be publicly available for all stakeholders, with adequate protection of any intellectual property rights for the developers of the models. If making the models publicly available is not feasible, at a minimum, the model(s) must be available to the manufacturer.

FIFARMA's position on Stakeholder Involvement:

- In order to meet the needs of patients, the system should have appropriate mechanisms to enable

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the public, including manufacturers and relevant patients and patient groups, to provide input. Other stakeholders that should be consulted include relevant medical experts whenever appropriate. Draft (i.e. not final versions) assessments should be made available for comment by, at a minimum the manufacturer, and ideally all relevant stakeholders.

- In addition, an oral hearing should be conducted where manufacturers can express their opinions on the draft HTA outcome, and other relevant stakeholders should also be able to participate in this hearing.
- Any decisions arising from the evaluation should be freely accessible to all stakeholders. Reports of the decisions should include a detailed discussion of the basis for conclusions, while protecting any business confidential information used to reach a determination.
- If the manufacturer's comments are not incorporated into the final assessment, the HTA body should be required to explain their rationale for the exclusion.

FIFARMA's position on Independence of Assessment:

- The HTA body must be independent from the reimbursement authority to avoid bias in the assessment process. The HTA body's primary goals should be to optimize patient access to effective treatments and to protect patients from ineffective therapies. The focus should not be on cost containment. Hence, independence of the HTA body is of great importance to assure a quality, transparent and unbiased assessment outcome.
- If an HTA body uses an external group for partially or entirely conduct assessments, this group should be duly accredited by the government to perform such an evaluation.

FIFARMA's position on Appeal Process:

- Following publication of the health technology assessment results, affected parties should have the right to appeal. Processes need to be in place to ensure efficient and independent handling of appeals.
- The grounds for appeal should extend to a different interpretation of evidence. The body hearing the

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appeal should be an independent, administrative body that includes experts who were not involved in the initial HTA process.

- Among the rational for appealing an HTA decision should include the fact that the HTA body acted biased, that the decision cannot be justified in light of the evidence submitted, or that the HTA body did not go through all the formal process.
- This appeal should be conducted in a timely and expedited manner.

IX. Quality Assurance of HTA.

- Due to budgetary and structural constraints, HTA bodies must often choice to outsourcing or alliances with third parties (both public and/or private) to develop partial or total health technology assessments.
- Being this an attractive market, and not having enough people with real training and experience in health economics and health technology assessment in Latin America, not all people or companies that offer these services to HTAs are suitable, and some are not independent

being aligned with the interests of one or more actors in the system.

- In some cases vendors with unclear skills to develop analysis are contracted and their analyses are used to take decisions in the health care system.
- In order for an HTA to be most effective, the health care system should be capable of delivering safe, quality care to its consumers in an organized, consistent manner.
- Ideally, the HTA system must be fully-staffed by well-trained professionals with the appropriate professional skill sets, including clinicians, policy analysts, economists, academics, and epidemiologists.
- The HTA system should be robustly funded and sustained along with adequate, publicly transparent oversight.
- Generally, it is preferable to refrain from integral transferability of foreign HTAs which include price and valuation that may not be suitable for the importing system. However, in some cases, it could be appropriate to transfer selected portions of an HTA outcome.

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- Health technology assessments that are not conducted by the HTA body itself but are contracted to third parties should be restricted to individuals and vendors that are accredited.
- In order to guarantee a certain level of quality for the health technology assessment, the accreditation should be based on the implementation of a minimum set of qualifications and experience.

SUMMARY

- *FIFARMA recognizes that HTA has emerged globally as the preferred discipline through which governments and payers are being recommended in their decisions about whether or not to pay for a technology (access and coverage) and how much (reimbursement).*
- *This has occurred in response to rising concerns about the high and growing costs of healthcare, in particular pharmaceutical expenditures.*
- *As a key stakeholder in any HTA-based pricing and reimbursement system, Pharmaceutical Industry is responsible of finding an effective manner in which **to partner with governments** to shape both the design and ongoing application of HTA processes in order **to achieve the highest level of opening and transparency, consultation and participation, fairness and credibility, predictability and independence, oriented to value.***