Defining the Scope of Health Technology Assessment and Types of Health Economic Evaluation

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Health Technology Assessment (HTA) is a process that uses principles from across various disciplines, including medicine, sociology, economics, and ethics, to evaluate health technologies. Policy makers can use HTA as a tool to assess health technologies in a systematic, unbiased, transparent, and robust manner in order to make informed and evidence-based decisions. Generally, researchers begin an HTA by defining the overall scope of the assessment, after which they choose an appropriate type of health economic evaluation, one of the most important elements of HTA. The objective of this article is to provide recommendations about the scope of HTA as well as guidance on the kinds of health economic evaluation that are appropriate in the context of the new Thai HTA guidelines. A well-defined research question that addresses five major components–target population, technology or intervention, comparator, outcome of interest, and perspective–is an essential part of any HTA.

Keywords: *Health technology assessment, Scope of health technology assessment, Health economic evaluation*

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Health Technology Assessment (HTA) is a process that uses principles from across various disciplines, including medicine, sociology, economics, and ethics, to evaluate health technologies. Policy makers can use HTA to assess health technologies in a systematic, unbiased, transparent, and robust manner, so that they can make informed and evidence-based decisions. In recent years, four main areas have emerged among those interested in HTA–policy analysis, evidence-based medicine, health economic evaluation, and social and humanistic impact assessment[1]. However, while these areas have been widely accepted, it is important also to acknowledge that not every HTA will necessarily include consideration of all four.

HTA is usually initiated at the beginning of a policy process so that the findings can be used to inform at the various steps of the process e.g. agenda setting, policy formulation, decision, and evaluation. In this way, policy makers are able to use HTA to help them make evidence-based decisions and, eventually, to ensure that cost-effective technologies are given priority over those which have doubtful value for the health system. In the last twenty years, the importance of using evidence-based decision-making when assessing the efficacy or effectiveness of health technologies has been increasingly recognised. In general, the evidence that is used in HTAs is experimental, quasi-experimental, or comes from observatory studies. Recently, comparative effectiveness data from head-to-head clinical trials or comparisons using both clinical and management data, has been used to inform clinical guidelines and health insurance benefit coverage guidelines. However, these kinds of evidence usually do not include information on the cost-effectiveness of the technologies under consideration. This is where health economic evaluations can help. Health economic evaluations assess the trade-off between the resources necessary to adopt a certain technology and the benefits of that technology once it is adopted. In essence, an HTA investigates the extent to which the adoption of a specific technology is cost effective. While economic evaluations are only one part of HTA, they have historically been the aspect that has been focused on the most. However, it is important to recognize that HTAs involve more than just an assessment of the benefit of a technology for an associated resource use. HTAs should include not only an assessment of the medical evidence behind and an economic evaluation

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of the technology in question, but also the impact of that technology on organisations, society, ethics, and humanity[1,2].

All HTAs should begin by defining the overall scope of the assessment and the research framework that will be utilised, based on general HTA concepts and principles. For instance, a clear HTA research question should be defined, based on the policy context and a review of the existing literature. It is also important that researchers clarify the perspective that they will be adopting in their research. Since health economic evaluations are a major part of any HTA, this article briefly defines the four main types-Cost-Minimisation Analysis (CMA), Cost-Benefit Analysis (CBA), Cost-Effectiveness Analysis (CEA), and Cost-Utility Analysis (CUA). The basic concepts of the incremental cost-effectiveness ratio (ICER) and the cost-effectiveness plane are also presented.

Concepts and principles of HTA

This section intends to provide an overview of the general concepts and principles of HTA. It gives guidance on the scope of HTA, provides details on the kinds of research framework that are used and the principles that lie behind technology selection. It also provides a summary of the types of health economic evaluations that are used in HTA and some guidance on how to interpret HTA results.

Defining the scope and research framework

Health technology selection for HTA

In any healthcare context, it is usually not possible to assess all health technologies due to resource limitations. Indeed, only some health technologies will be appropriate for HTA. Generally, the selection of a technology for assessment depends on the priorities of the organisation undertaking the assessment. However, there are two important principles that should always inform the decision when selecting a health technology for assessment to be rigorous, evidence-based, and transparent[3-5].

Selection criteria: Although most countries do not have a well-defined set of criteria to help guide health technology selection for HTA, there is widespread consensus that any technology that has been shown to offer significant health benefits or impact significantly on health-related policy e.g. in terms of equity or patient access, is worthy of assessment. Moreover, most countries agree that consideration should also be given to those technologies that treat diseases that are particularly virulent and could create major financial losses to either individuals or society. Finally, there is also agreement that technologies that have a particularly wide application and those that are in particularly high public demand should also be considered for HTA.

Selection process: After selection criteria are identified, different countries have their own different selection processes, e.g. priority setting by scoring system, expert opinions, etc. However, transparency and participation are common principles of health technology selection for HTA.

Defining the research question

In essence, HTA is a policy research tool[6]. The first step in undertaking an HTA therefore, is to decide which research question is suitable, given the policy question under discussion. Usually, well-defined research questions emerge from a collaborative discussion between stakeholders and researchers. A good research question will always include five components[7,8].

Target population: This defines the population of interest by defining various characteristics. The characteristics that are specified should always include age, gender, and disease risk, as a minimum.

Technology or intervention: This will specify the type of technology under discussion. Details on how innovative the technology is, will be given here and any existing alternatives to the technology should be outlined.

Comparator: Similar details indicated for technology or intervention, which is chosen to be compared with the technology or intervention of interest, should be provided.

Outcome of interest: The expected outcomes should be clearly indicated.

Perspective: The type of perspective that is to be used should be indicated.

In addition, a well-defined research question should include an outline of the overall research context within which the assessment is taking place-i.e. by defining who is calling for the HTA, identifying the primary stakeholders (policy makers, health care providers, patients, etc.), and clarifying how this HTA is expected to be beneficial.

Health technology background review

Reviewing the existing literature and information on the condition and treatment under investigation is an important part of any HTA.

Researchers should present a clear contextual description of the condition, including an outline of its consequences, prognosis and progression, clinical characteristics, period, and treatment alternatives. Researchers should always be aware that their readers may not be clinical experts. Researchers should also provide an overview of any other background information that will be relevant to the HTA, including summarising any safety issues that have emerged, as well as providing an overview of the existing data on the health technology's efficacy and effectiveness. The details of the outcomes and its measurement should also be clearly described.

HTAs should always include a health technology background review–not only for technologies such as pharmaceuticals, but also for medical devices, community interventions, and other medical procedures. The health technology background review should examine both the technology itself, as well as relevant factors that affect its implementation e.g. facilities, procedures of care, distributions of technology, indications, price, legal issues, manufacturers, market shares, etc.

Target population

In the target population section of the HTA, researchers should begin by providing an overview of the disease's epidemiology, including prevalence and incidence. This helps to give a general idea of the extent to which the disease condition affects the general population. The target population for the HTA should then be clearly defined, including their age, gender, socioeconomic status, and risk factors. This means that the results of the study can be linked clearly to the appropriate population, rather than the general population, which is important in determining the extent of cost-effectiveness (i.e. whether a treatment is cost-effective for the general population, or only to the target population). These kinds of subgroup analysis also help to reduce bias.

Selection of comparators[9]

The selection of appropriate comparators is also an important step when undertaking an HTA, especially in terms of the health economic evaluations. Having appropriate comparators helps guide how the HTA results should inform practice and prevents bias, by ensuring that relevant alternative technologies are also assessed for cost-effectiveness. Any technology used in current practice, which is agreed by the relevant stakeholders, can be a comparator. While comparators are often technologies that are direct alternatives to the technology under consideration, all possible alternatives should be considered and assessed until the most appropriate comparators are found. Indeed, although the technologies that are most widely used in the treatment of the condition under examination are often used as comparators, they may not always be the best choice, particularly if the widest-used technology is not the most effective technology. The technology that has been shown, to date, to be most effective (especially those that are recommended by standard practice guidelines and those recommended by experts), should be used as a comparator, where possible. It is also important to note that the watchful waiting option (WAW), rather than implementing an alternative is often the most appropriate comparator. There is no restriction on the number of comparators that can be included in an HTA; the number will depend on various limitations, including data and time availability.

Perspective[9]

Defining the perspective that the HTA will adopt is an important part of the HTA process, since this will define, to a certain extent, the different costs and outcomes of the assessment. For instance, the broadest societal perspective will take into account the direct and indirect costs of both the health care system and the patients. In contrast, the narrower perspective will focus only on the costs of one body–e.g. a health ministry or a hospital, and will exclude all patient costs.

Even though there is no such thing as a "best perspective", researchers should define their perspective based on the objectives of the HTA user. In general, however, broader perspectives tend to give more informative results, which maybe more useful for those involved in policy decision-making.

Health economic evaluation methods

Types of health economic evaluation

Limited health care budgets mean that decisions about the amount of resources that can be allocated to health technologies have to be made. In policy decision-making, health economic evaluation is an important tool that is used to compare various treatment alternatives to ensure that policy-makers are able to allocate health care resources in the most efficient way.

A health economic evaluation is a comparative analysis of a number of treatment options. It focuses

on two factors–costs and outcomes[8]. Drummond et al have identified four types of health economic evaluation: Cost-Minimisation Analysis (CMA), Cost-Benefit Analysis (CBA), Cost-Effectiveness Analysis (CEA), and Cost-Utility Analysis (CUA). Table 1 summarises the cost and outcome definitions and measurements of these four types.

Cost-minimisation analysis (CMA)

CMA is an analysis that compares two or more alternatives that have demonstrated equivalence. It is used to show the alternative that has the lowest costs[7]. While useful, there have been some criticisms levelled at the approach. For instance, Briggs et al identified relatively high levels of uncertainty in the process of cost and outcome estimations[11]. This means that the only technologies that can be meaningfully compared using CMA are those that are really equivalent. Given this, Drummond et al have recently suggested that CMA be used only as a cost analysis tool, rather than a full economic evaluation[8].

Cost-benefit analysis (CBA)

CBA is an analysis that compares technologies by converting outcomes to monetary units and then comparing the costs and resources used (*e.g.* by calculating amounts such as the costs saved as a result of using the technology of interest, and the costs that were avoided as a result of early diagnosis). In some CBA cases, the willingness-to-pay (WTP) of patients is included, so that the analysis is informed by the maximum amount of money that patients would be happy to pay to treat or prevent the condition[8,10]. Since both costs and outcomes are measured in monetary units, any technology where the costs are found to be lower than the value of outcome is cost-effective. This method allows easy comparison between two or more technologies, as all costs and outcomes are converted into the same monetary unit. However, the conversion of health outcomes to the monetary unit is challenging and controversial. It limits the use of CBA in health economic evaluation.

Cost-effectiveness analysis (CEA)

CEA is widely used in health economic evaluations to ascertain the most efficient way to allocate resources. This approach measures costs in monetary units and outcomes in efficacy or effectiveness units[10], (e.g. cases of correct diagnosis, deaths, life-years gained, etc.) and then compares the value of a given treatment, given these measurements. Outcomes can be quite specific (e.g. diastolic blood pressure reduction, reduction in the number of pain points, etc.), which allows for quite detailed tailored analysis. Intermediate outcomes (e.g. detected risk factors) and final outcomes (e.g. cases prevented) are also sometimes included. However, the use of intermediate outcomes in CEAs is not recommended, since this may result in biased interpretation of study results due to its limited use in predicting final outcomes. A major limitation of CEA is that it cannot be used to compare health technologies with different outcomes.

Cost-utility analysis (CUA)

CUA is used when the outcome of interest is health-related quality of life. The most widely used outcome measurement in CUA is the quality-adjusted life year (QALY), which captures both quantity (life

Table 1. Types of Health Economic Evaluation[8,10]

Type of analysis	Cost measurement	Outcome characteristics	Outcome measurement
Cost-minimisation analysis	Monetary unit	Every outcome is equivalent.	Assuming equivalent out comes, measurement is not necessary.
Cost-benefit analysis	Monetary unit	Every outcome may not be the same because it must be converted to monetary unit.	Monetary unit
Cost-effectiveness analysis	Monetary unit	Every outcome must be in the same unit.	Efficacy e.g. life years gained, number of correctly diag nosed patients, reduced blood pressure, etc.
Cost-utility analysis	Monetary unit	Every outcome may not be the same because it must be converted to utility.	Quality-adjusted life years (QALYs)

year) and quality (quality of life as a utility measurement) of outcomes. In general, utility score varies from zero (death) to one (full health).

To undertake a QALY calculation, the utility score of a health state would be multiplied by the time the patient spent in that state. For instance, if a patient has full health for the first six months (utility score 1), followed by six months where they suffer from condition Z (utility score 0.5), this patient would have gained a 0.75 QALY.

Tarride et al found that the methodology that is used to determine utility scores or health preferences is an important part of CUA[10]. Generally, there are two measurement methods–direct and indirect. Standard gamble (SG) and time-trade off (TTO) are examples of the direct method, but they use a lot of resources and can be time-consuming to undertake. Euro Qol (EQ-5D) and Health Utility Index (HUI) are examples of the indirect method. QALY is not only the measurement outcome that is used in CUAs. Another commonly used outcome is the disability-adjusted life year (DALY)[8], which was developed by the World Bank and Harvard University and, subsequently adopted by the World Health Organisation (WHO) in 1996.

Incremental cost-effectiveness ratio (ICER)

While choosing the appropriate type of health economic evaluation is an important part of HTA, the way that results are interpreted is also important. It would, of course, be easy to make decisions regarding superior treatment if all new technologies were superior to older ones in terms of better health outcomes and cheaper costs. However, the reality is that, while most new technologies tend to give better health outcomes, they also come with higher costs. Therefore, policy-makers need to make a trade-off between costs and health outcomes. Tarride et al has suggested that the goal of any health economic evaluation is to determine how much more it is appropriate to pay for a unit of a health outcome, when a new technology replaces an existing technology[10]. The incremental cost-effectiveness ratio (ICER) is an effective way to measure this; it is based on a calculation that determines the difference between the costs of old and new technologies, divided by the difference between their outcomes. The formula for determining an ICER value is shown below:

$ICER = (Cost_A - Cost_B)/(Outcome_A - Outcome_B)$,

where A represents higher cost and better outcome technology, and B represents lower cost and poorer outcome technology. For instance, if a new technology and an old technology cost 200,000 Baht and 140,000 Baht, respectively, and the new technology results in a gain of 1 QALY while the old technology results in only a 0.5 QALY gain, then the ICER value will be 120,000 Baht ((200,000-140,000)/(1-0.5)) per QALY. This means that, in this case, the new technology will cost 120,000 Baht per QALY more than the old technology for equivalent outcomes. Another kind of measurement that is sometimes used to calculate results in an HTA is the average cost-effectiveness ratio (ACER). It is often confused with ICER. The main differences between the two types of equation are given in Table 2 below.

In health economic evaluations, ICER is used to indicate whether a technology is cost-effective or not. The ACER, on the other hand, is not appropriate for making these kinds of decisions, since the ratio compares the technology of interest with a do-nothing alternative, which has no cost and no health outcome. In practice, WAW is rarely a good comparator, since it not only is it rarely used in the practical healthcare setting, but its usage can also be ethically problematic. In addition, WAW often generates more costs, since the lack of treatment can result in more severe symptoms and more costly in the future.

Another tool that is useful in conducting health economic evaluations is the cost-effectiveness plane[10]. This is a graphical plane between the incremental cost and incremental outcome. If we apply this to the above-mentioned example, then the line would be plotted as shown in Fig. 1. The horizontal axis represents the incremental outcome while the vertical axis represents the incremental cost between new and old technologies. The cost-effectiveness plane has four quadrants–A, B, C, and D. Quadrant A is the area of the ICER where the old technology is the dominant alternative, while quadrant D is the area where

Table 2. ACER and ICER Examples

	Cost(Baht)	QALY	ACER	ICER(Baht/QALY)
A	200,000	1	200,000/1	(200,000-140,000)/(1-0.5)
B	140,000	0.5	140,000/0.5	

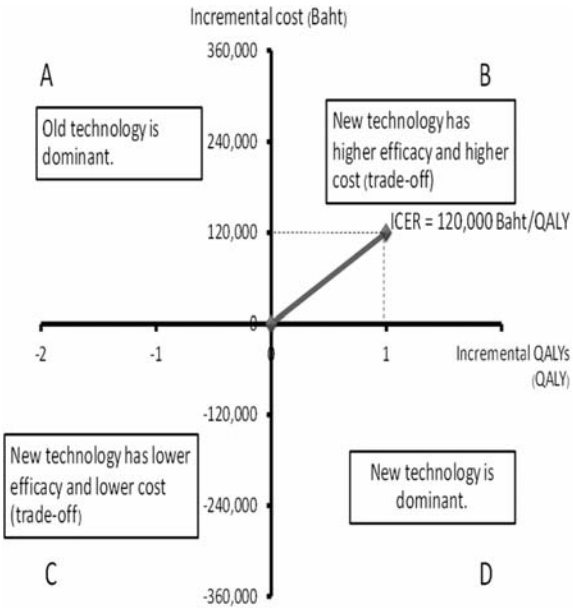

Fig. 1 Cost-effectiveness plane (adapted from Drummond et al)[8].

the new technology is the dominant alternative. Quadrant B and C are areas that show the trade-off between costs or resources used and outcomes obtained since they are the areas where the new technology has higher costs and better outcomes or lower costs and poorer outcomes. The cost-effectiveness plane can help policy-makers quickly understand the various benefits and drawbacks of a new technology and its comparator.

Guidelines for health technology assessment in Thailand (second edition): Recommendations for defining the scope of HTA

Given the concepts and principles of HTA and health economic evaluations that have been presented here in, several recommendations are made for researchers in terms of defining the scope of HTA and the types of health economic evaluation that are appropriate for the Thai context.

1. All HTAs should include a well-defined research question that incorporates five major components–target population, technology or intervention, comparator, outcome of interest, and perspective.

2. All HTAs should include a comprehensive background information review that summarises all relevant information on the disease and health technology in question.

3. The target population in every HTA should be clearly described.

4. All comparators should be technologies that are used in current practice, and which have been discussed or approved by stakeholders. They should also be technologies that may be replaced by the technology of interest. Regardless of the type of comparator that is used, clear reasons and details must be provided.

5. The societal perspective should be adopted.

6. A Cost-utility analysis (CUA) is recommended to be the method of choice. If data or resources are limited, CEA can be used. However, the use of intermediate outcomes is not recommended.

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Potential conflicts of interest

None.

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การกำหนดขอบเขตการประเมินเทคโนโลยีด้านสุขภาพและวิธีการประเมินความคุ้มค่าทางสาธารณสุข

พิมพ์วรา ตันเวชศิลป์, สุรฉัตร ง้อสุรเชษฐ์

การประเมินเทคโนโลยีด้านสุขภาพเป็นกระบวนการที่เกิดจากการนำศาสตร์จากพหุสาขา ได้แก่สาขาทางการแพทย์ สังคมศาสตร์ เศรษฐศาสตร์ และจริยศาสตร์ ที่เกี่ยวข้องกับการใช้เทคโนโลยีด้านสุขภาพมาใช้อย่างมีระบบ โปร่งใสและปราศจากการมีอคติ เพื่อให้ได้เป็นข้อมูลที่มีประโยชน์ในการสนับสนุนกระบวนการตัดสินใจเกี่ยวกับเทคโนโลยีด้านสุขภาพ ร่วมกันกับเหตุผลและปัจจัยอื่นๆ ของผู้มีหน้าที่ตัดสินใจเชิงนโยบายในการประเมินเทคโนโลยีด้านสุขภาพ นักวิจัยควรเริ่มด้วยการนิยามขอบเขตของการประเมินและการเลือกวิธีการ ในการประเมินความคุ้มค่าทางสาธารณสุข ซึ่งเป็นองค์ประกอบหลักของการประเมินเทคโนโลยีด้านสุขภาพ ดังนั้นวัตถุประสงค์ของบทความนี้คือการให้คำแนะนำเกี่ยวกับขอบเขตของการประเมินและวิธีการในการประเมินความคุ้มค่าทางสาธารณสุขโดยยึดแนวคิด และทฤษฎีที่เกี่ยวข้องเป็นหลักในการประเมินเทคโนโลยีด้านสุขภาพ นักวิจัยควรมีการกำหนดคำถามงานวิจัยที่ชัดเจนซึ่งประกอบด้วย 5 องค์ประกอบหลักคือกลุ่มประชากรเป้าหมาย เทคโนโลยีที่จะประเมิน เทคโนโลยีที่ใช้เปรียบเทียบ การกำหนดผลลัพธ์ที่สนใจ และมุมมองที่ใช้ในการประเมิน