

Economic Evaluation in Health Care

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This resource replicates contents from the Online Certificate Course on Use of HTA in Decision Making, Module 6A - "[Health Economics for HTA. Introduction to Health Economic Evaluation](#)" provided by the Institute for Clinical Effectiveness and Health Policy.

1. Structure of Economic Evaluations

Introduction to the Theoretical Framework

It is desirable to determine the objectives and limitations of the studies. This involves specifying:

- The main question
- The analytical approach
- Comparison options
- Costs and results included

Early evaluation of these aspects is paramount, since it affects data collection, the way these data are analyzed and the way they are interpreted. If an inappropriate choice is made with regard to the point of view, the comparators, or the follow-up period, this would lead to an incorrect conclusion that could yield, at the very least, inadequate research, and in the worst-case scenario, it would lead to an inappropriate decision.

Choices should be based on the form and context in which the economic evaluations are to be used. All assumptions, the way in which the data are collected, and the sources used must be unequivocally and precisely recorded. It is important to consider how the sensitivity analysis will be done, what the original contributions of the work are, as well as its limitations. The key aspects for an economic evaluation to be comprehensive and accurate are:

- The purpose of the analysis
- The evaluation audience
- The Economic evaluation Perspective
- The analytical horizon
- The specific intervention being analyzed
- The alternative intervention for comparison
- The target population

We can thus rest assured that the resulting economic evaluation is appropriately structured and can be interpreted within its context.

Comparative Nature of Economic Evaluations

Economic Evaluations are always useful as comparisons of different options. Comparisons can be made between two or more interventions, usually comparing new interventions versus the current state of the art, the usual practice. We must include the most significant costs and outcomes of all the options evaluated.

The simplest way to make comparisons is to take the results of the intervention being studied and evaluate them directly in their natural units, such as reduced blood pressure millimeters of mercury, kilograms of weight lost, days of hospitalization avoided, or years of life gained. This is not always possible, as we often have to compare between interventions whose results are expressed in different units: how do we determine whether we should invest in the treatment of hypertension or obesity? In the first one we have a reduction of millimeters of mercury and in the latter, kilograms lost.

When we intend to compare interventions applied to different pathologies, we must obtain a common result for both, for which we will develop the concept of QALY and DALY. That is how we can extend the scope of the comparison.

Regardless of whether our objective entails comparisons between projects with little in common, restricted only to resource consumption and the search for the well-being of the population, we can theoretically “convert” the units of measurement of the benefits into money and thus cover virtually all areas that are relevant to decision makers.

Types of Economic Evaluations

There are different types of economic evaluations, depending on how health benefits are measured.

For that purpose, the following types can be contemplated:

- Minimization-cost analysis
- Cost-effectiveness analysis
- Cost-utility analysis
- Cost-benefit analysis

All complete economic analyses use the same cost identification, measurement, and valuation. The difference lies, however, in the way they measure the outcomes.

1.1. Cost-minimization analysis

In these studies, both alternatives yield the same result, that is, there are no effectiveness differences (they are equi-effective). It should be noted that effectiveness must be considered broadly and does not refer to a single clinical outcome alone. For example, if two drugs are equally effective in their ability to reduce mortality from a given condition, but one of them causes severe adverse effects, then they cannot be said to be equivalent.

The main goal of cost-minimization studies is to find the least expensive alternative, given that the consequences are the same.

Cost-minimization analysis is used when comparing two interventions that have been shown by previous studies to have equal efficacy and effectiveness.

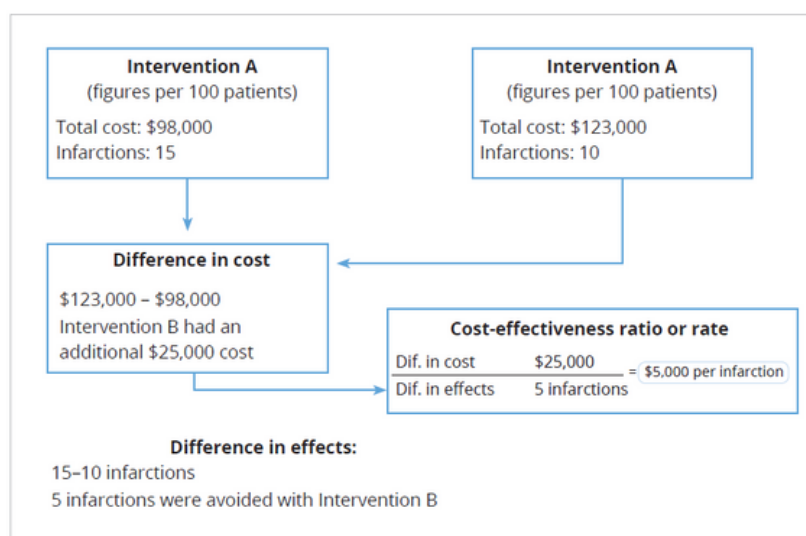
An additional use of these studies is to identify the distribution of costs and explain why an alternative is less expensive. For example, myopia corrective surgery can be performed on an outpatient or inpatient basis. Both alternatives are equally efficient and effective but the first one is less expensive. These evaluations are seldom found since there are few equi-effective interventions.

1.2. Cost-effectiveness analysis

In this type of analysis, benefits are measured in natural units. Some examples can be years of life gained, blood pressure millimeters of mercury dropped, kilograms of weight lost, breast cancer cases detected, or infarctions avoided. In this case, the benefit assessment measure is common to both options. To compare them, their differences, both in costs and effects must be estimated, which will allow us to calculate cost-effectiveness ratio or rate.

Let's examine an example where two preventive treatments to avert heart attacks in a group of high-risk patients are compared.

The results obtained are as follows:



In this example, the costs included in the analysis are not only the costs of the treatment itself but the cost of care for infarction patients. Perhaps treatment B was individually much more expensive than treatment A, though it also provided savings due to the infarctions avoided. In any case, we see that these savings failed to offset the difference in cost of the treatments, so Intervention B continued to be more expensive and its cost-effectiveness, compared to treatment A, turned out to be \$ 5,000 per infarction avoided. In other words, with intervention B, it is necessary to invest \$ 5,000 to avoid an infarction. This result already includes the costs of treatment and the savings generated by the infarctions averted, so it is the net result of the additional investment needed to achieve the health benefit of avoiding an infarction.

In cost-effectiveness studies, a natural parameter common to both alternatives being evaluated is selected. They do not allow to compare options without a common result.

Cost-effectiveness studies are very useful, though they do have some drawbacks. The two main disadvantages are mentioned below:

1. There may be interventions leading to more than one clinical outcome (very common), so the end result cannot be combined into a single value.
2. Cost-effectiveness studies do not allow to compare interventions from different areas, or even from similar areas if different cost-effectiveness measures are chosen.

Example

Let's assume a health decisionmaker needs to decide on four new programs to be incorporated into the following year's care in his or her region:

- 1. A breast cancer detection program improvement: cost-effectiveness of \$1,870 per additional breast cancer case detected.*
- 2. An infarction prevention program: cost-effectiveness of \$4,000 per infarction averted.*
- 3. A blood pressure control program: cost-effectiveness of \$22 per blood pressure millimeter of mercury dropped*
- 4. A flu vaccination program: cost-effectiveness of \$1,000 per avoided hospitalization.*

How can interventions leading to different results be compared, then chosen?

It is partly for these reasons that cost-effectiveness studies have gradually been replaced by the category we will examine next: cost-utility studies.

1.3. Cost-utility analysis

In cost-utility studies, the preferred type of EE today, the way consequences are measured is a combination of years lived and quality of life. This type of analysis is used for three main reasons in different situations:

- When interventions have multiple consequences, all of which are of interest to us. For example, in the treatment of sedentary lifestyle we may be interested in evaluating physical training, kilograms of weight lost, cardiovascular prevention (number of infarctions prevented, years of life gained), improvement of dyslipidemia, reduction of depression and anxiety.
- When we are interested in obtaining a result that allows us to compare alternatives that are not related to each other and do not have the same consequences. For example, we need to decide on or compare the cost-utility of a malnutrition treatment to other unrelated interventions such as the treatment of infarction.
- When we are particularly interested in measuring both survival and the quality of life that this survival entails.

The great advantage of cost-utility studies is that they allow us to combine all intervention effects into a single value.

Economic Evaluation in Health Care

A particular treatment can extend life, prevent hospitalizations, and improve a symptom, but cause a serious adverse effect or some complication at the same time. All these effects can be combined into a single measure, QALYs (Quality-Adjusted Life Year), and this value can be used to compare the treatment to another one, which can also have, in turn, multiple effects, even different ones, that can be similarly summarized.

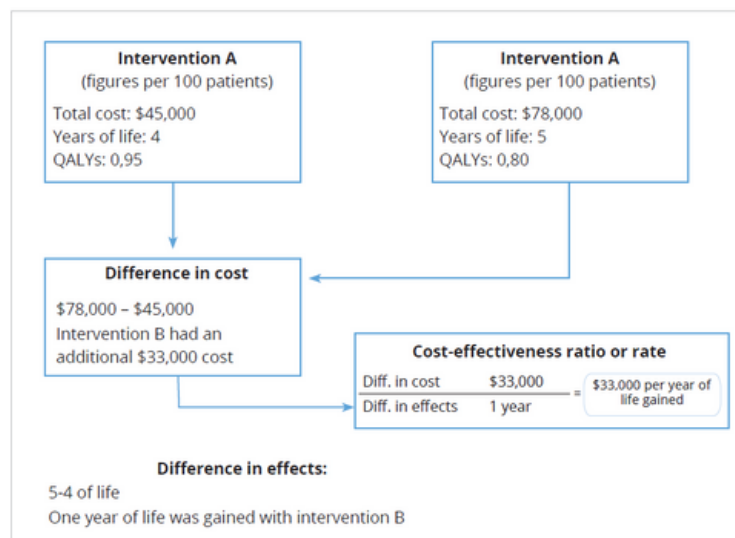
Now, what does QALYs mean and where does it come from? A brief explanation on this point is presented below.

QALY is a measure to express the equivalence of a year of life with a certain condition. The “unit” used for this comparison is a year lived in full health. For example, if we say that the QALY of renal dialysis is 0.65, we mean that living one year with Chronic Renal Failure (CKD) requiring renal dialysis equals 0.65 years in full health:

1 year on kidney dialysis = 0.65 years in full health.

Why? Because kidney dialysis affects quality of life; therefore, a year on dialysis is “worth” less than a year without dialysis. This has significant implications when evaluating the cost- utility of interventions.

Let’s consider an example where two prostate cancer treatments are compared, one of which has the advantage that it can extend life, though at the expense of causing certain important adverse effects (such as urinary incontinence or impotence). The results obtained are as follows: In the first case we will compare the interventions using a cost-effectiveness study, then we will analyze them from the perspective of a cost-utility study.



As seen above, in this first cost-effectiveness analysis, we only took one result into account: life extension. We did not consider the differences in quality of life between the two interventions. To take both factors into consideration we must use QALYs. As can be seen, treatment A achieves a 4 year survival period with an average quality of life equivalent to 0.95 QALYs. Therefore, these 4 years of survival are equivalent to 3.8 years in full health or QALYs (this is the result of multiplying the duration of life -4 years- by its quality -0.95-). Treatment B achieves a longer survival, 5 years, but due to its adverse effects the quality of life is lower: 0.80 QALYs. Therefore, these 5 years of survival are equivalent to 4.0 years in full health or QALYs (this is the result of multiplying the duration of life -5 years- by its quality -0.80-).

A cost-utility study will use this measure to calculate the cost-utility ratio or rate. From this perspective, the difference between both interventions is no longer one year of life but 0.2 QALYs (4.0 QALYs with treatment B – 3.8 QALYs with treatment A).

| Cost-effectiveness ratio or rate | | |
|----------------------------------|---|----------------------|
| Diff. in cost | = | \$33,000 |
| Diff. in effects | = | 0.2 years |
| | | = \$165,000 per QALY |

It is more evident now that the difference between the two interventions is not as important as it first appeared to be, since the life extension in one case is offset by the improvement in the quality of life in the other.

As seen, then, cost-utility studies offer great advantages since they allow several different effects to be incorporated into a single result. In addition, by expressing their results in a common unit, they allow to compare interventions from different areas.

Do you want to know where the QALYs figures are obtained from?

This question is more complex, and a detailed explanation exceeds the scope of this class, but we will try to comment on it briefly.

To know the “value” or “utility” that a person gives to a certain state of health we could use the following method. We can describe to this person the state of health, for example kidney dialysis, and ask him/her how long in full health he would be willing to change for a year on kidney dialysis. Suppose that this person tells us that living one year on kidney dialysis would be equivalent to living eight

months in full health (because the person is obviously willing to sacrifice a certain period of life, 4 months in this case, in exchange for improving its quality). With this information we could estimate what the “equivalence” is for this person:

8 months in full health = 12 months on dialysis $\rightarrow 8 / 12 = 0.67$ QALYs.

In other words, for this person, a year on dialysis is equivalent to 0.67 year in full health.

We could apply this very evaluation to several people representative of a population, and we would thus obtain an average value for this QALY. This method of calculating QALYs, which we have just briefly described, is called TTO (acronym for Time Trade-Off) because it is based on an estimation of how much of his/her life a person would be willing to sacrifice to become fully healthy.

TTO is just one of many existing methods for calculating QALYs. No method is unanimously considered better than others and there is much controversy on this subject, but at least the example serves to briefly illustrate one of the methods of obtaining these values.

Let's go back to the example we saw a while ago...

Now suppose that the cited decision-maker receives this information expressed in terms of cost-utility:

1. A program to improve breast cancer screening: cost-utility of \$55,000 per QALY.
2. An infarction prevention program: cost-utility of \$45,000 per QALY.
3. A blood pressure monitoring program: cost-utility of \$120,000 per QALY.
4. A flu vaccination program: cost-utility of \$220,000 per QALY.

Now we can compare these results and know that b (prevention of infarction) is the most “cost-effective” of all these programs. According to this program it is necessary to invest \$ 45,000 to achieve a year of life in full health, a result that is better than those offered by the rest of the interventions.

For these reasons, economic evaluations are now preferred to be cost-utility studies, unless there are good reasons to choose another type of design. However, the term “cost- effectiveness” is so widespread that it is common to generally refer to EEs as cost-effectiveness studies, this term encompassing both actual cost-effectiveness studies and cost-utility studies.

In Cost-Utility analyses, QALYs (Quality-Adjusted Life Years) are used as a measure of outcomes.

In this case, clinical results are translated into monetary units. It requires that a monetary value be attached to the consequences. This topic is very controversial. How much is life worth? It is a concept that is not pleasant to doctors or the general population.

Cost-effectiveness analysis measures the result in monetary units.

It is worth noting that in the previous analyses it is an all or nothing issue, meaning that the entire budget will be allocated to one or another option excluding everything in between. In everyday life, however, most often there are situations where we want to allocate a portion of the budget to one project and another portion to another. For example, if we have a \$100,000 budget, we can invest 40% in one of the alternatives and 60% in the other.

Objective of the analysis of an evaluation

Just as the objective of a clinical trial is to evaluate what is the most effective treatment for a given condition, the objective of an economic evaluation is to evaluate the most efficient way to use the available resources. While this definition appears to be simple, certain characteristics of economic evaluations that allow its interpretation should be considered:

- The audience to whom it is addressed.
- The context
- The comparators

1.4. Economic evaluation audience

It is important to consider, whenever an economic evaluation is performed or commissioned, who its recipients will be, that is, its users. They will be, essentially, all decision-makers for whom the information provided by the evaluation is useful.

This audience may be very different from the people who conducted the research, and may include, among others:

- Governments (national, provincial, municipal). For example, Ministries of Health or Departments of Health, Commissions for the incorporation of Health Technologies into the public system.
- Hospitals;
- Healthcare payer organizations, such as social security or private payers.
- International Organizations (WHO, PAHO).
- Multilateral aid agencies (e.g., World Bank or Inter-American Development Bank).
- Regional country alliances (e.g., MERCOSUR).
- Non-governmental aid agencies.
- Pharmaceutical Companies.

Each of these organizations may have different requirements and sometimes even different guidelines for the execution of economic evaluations. In some cases, evaluations will even add knowledge rather than provide new information to decision makers or will be aimed at particular interest groups. The audience of the study largely defines its perspective, as explained below.

It is extremely important to identify and know the economic evaluations audience in the design phase of the study, as it helps to decide the most appropriate methods, as well as the way results will be reported.

1.5. Perspective or point of view of the analysis: cost categories

This is a specific ingredient of economic evaluation, unknown to those coming from the healthcare area but better known to those from the economics or managerial area. The perspective or point of view of a study, refers to the place from which we contemplate evaluating the problem. It is not the same if we use the perspective of the president of a country, or of the Minister of Economy or finance, as doing that from the perspective of a hospital director or a private health insurance. It is essential to define the perspective of the study from the time it is designed, since it determines what type of costs need to be considered. For example, for a private health insurance company, the out-of-pocket costs that patients have to incur in order to attend treatments are not directly relevant. On the contrary, for a government decision-maker they may be relevant. Consequently, the perspective of a study mainly defines what types of costs will be included in the studies and which will not be considered.

Some of the possible perspectives are:

- patient
- physician
- hospital
- payer
- society

The vast majority of Economic Evaluations are performed from the point of view of the payer, although there is an ever increasing tendency to contemplate the social perspective.

The types of costs included in each perspective are the ones that are relevant to it. For example, a private health payer will include costs that pertain to him or her (outpatient health care costs, hospitalization, laboratory, etc.) but will not include other costs that are not his/her concern (e.g., transportation costs for patients and their families, or those related to lost labor productivity).

So, before we go further into the different perspectives, let's define the types of costs contemplated by economic evaluations.

The first distinction to be made is between direct costs and indirect costs (which have a different meaning in the field of economic evaluations than in the accounting literature). As the name implies, direct costs are all those directly related to the implementation of a certain program or the administration of a particular drug. In turn, direct costs are classified into:

- Direct costs of the health system (e.g. medical visits, nursing, drugs and other technologies, equipment, use of space);
- Direct costs of the patient and family (out-of-pocket expenses for transportation, non-professional hired help);
- Direct costs of other agencies or sectors (not as much covered in the economic evaluations). For example, an addiction prevention program would have a direct effect on reducing crime, thus lowering the costs of the legal or penal system.)

As for indirect costs, better called lost productivity costs, they refer to the valuation of the time that the patient must stop working due to the intervention being evaluated, or the time that a family member stops working due to this intervention (consider, for example, any pediatric treatment due to which parents or caregivers inevitably lose productive hours to accompany their children).

In the table below we can generally see the types of costs included for each of the US perspectives.

| Examples of cost | Study perspective | | | | |
|--|-------------------|--------|----------|-------|---------|
| | Patient | Doctor | Hospital | Payer | Society |
| Direct medical cost | Yes | Yes | Yes | Yes | Yes |
| Doctor's time | No | Yes | Yes | Yes | Yes |
| Other staff time (nursing, technicians) | Yes | No | Yes | Yes | Yes |
| Drugs | No | No | Yes | Yes | Yes |
| Medical devices and disposable supplies | No | No | Yes | Yes | Yes |
| Laboratory tests | | | | | |
| Direct non-medical cost | | | | | |
| Administration | No | No | Yes | Yes | Yes |
| Facilities/Building | No | No | Yes | No | Yes |
| Utilities (telephone, electricity, etc.) | No | No | Yes | No | Yes |
| Patient travel expenses | Yes | No | No | No | Yes |
| Temporary companion/caregiver | Yes | No | No | No | Yes |
| Indirect cost | | | | | |
| Time off work for doctor's visits | Yes | No | No | No | Yes |
| Time off work due to illness | Yes | No | No | No | Yes |
| Temporary help with household chores | Yes | No | No | No | Yes |

Then, if we conduct or read a study according to the payer's perspective, only the direct costs of the health system will be included, both those listed as medical costs in the table, and the administrative costs of the payer. It does not consider other costs such as lost earnings incurred by the patient, or the transport and caregiver costs. The social perspective is the most complete and comprehensive one. From such a point of view, all the resources consumed should be included in the study, no matter who consumes them, since all costs are relevant to society.

1.6. Time horizon

Analytical horizon refers to the period of time covered by the study. The following should be considered:

- Covering all the main costs and consequences of the pathology studied (a DBT study cannot last only 6 months)
- Known annual cyclical variations (more flu medicines are consumed in winter)
- Allowing the development or implementation and acceptance of a new technology (mechanical suturing took years to be refined and disseminated)

Following the previous example, hip prosthetic replacement surgery for osteoarthritis in our patient may imply a significant expenditure at an early time but great savings for the future both in medical visits and in consumption of specific analgesic medication. It follows then, that it is important to be clear about the time range covered by the evaluation.

If our time horizon is relatively distant (e.g., Economic evaluations of vaccination in children), then the analysis required will be more complex. In these cases, it may be necessary to use mathematical models to evaluate the costs and benefits at a future time for which we do not have primary data. We will expand on this issue in the next unit.

1.7. Specifying the intervention

It is necessary to specify and describe both broadly and in detail the interventions being analyzed and compared, as well as the health systems where these interventions are set. This helps to identify all the resources used and allows readers to understand exactly what is being analyzed, and also facilitates the generalization of the results. It is then easier for the reader to adapt the material to their local context, thus achieving a wider generalization with a greater impact of the work done.

The more complex the intervention, the more complex the analysis. However, it is important to emphasize the importance of evidence of the effectiveness of the interventions being analyzed, therefore some usual practices should be excluded. Bear in mind that reviewing many options complicates the analysis a great deal.

Various techniques have been developed to describe interventions, one of the most used and globally accepted of which is Drummond's list of topics to be described. It is suggested, in order to identify costs, that one should ask who does what, to whom, where and how often, and to identify the consequences one should inquire what the outcomes are. To answer these questions, researchers must consider healthcare-relevant activities as well as the establishment, monitoring, and control of new technologies and interventions.

WHO?

Human resources often involve a large share of costs that can vary greatly between countries and regions. The salary or pay is different between qualified and unskilled personnel, between a professional and an intern, an expert and a novice. Therefore, the roles must be well described in the analysis.

For example, care may be provided by doctors, nurses, aides, program-trained individuals, or community members. All interventions should be described, from the payer to the providers, in a way that helps identify the types of costs and data sources needed for the evaluation.

WHO DOES WHAT?

Describe all the activities associated with each intervention, this may include a clinical protocol and the training required for the implementation of an intervention or supervision visits.

FOR WHOM?

Personal characteristics of patients, such as age, sex, comorbidities, and risk factors, should be defined. If activities are divided into different groups (e.g., age or risk factor), each should be described separately.

WHERE?

It is important to explain where the intervention is carried out, for example at a health center or hospital. If the intervention is performed in different scenarios, this must be specified, together with a flow chart of the activities. Also state whether the intervention evaluated is autonomous or depends on or is related to other services (for example, smoking cessation depends on the clinical medicine or cardiology staff, family planning on the gynecology or family medicine staff). The description of the management and supervision of interventions should underscore the different levels of the health system involved. For example, a patient with cardiovascular disease who is cared for by his general practitioner (primary level of care) and is undergoing further studies in the hospital (secondary level of care) suffers an infarction and is admitted to the coronary unit for catheterization (tertiary level of care).

HOW OFTEN?

We must analyze:

- The period of time in which the intervention is expected to operate, for example one year, or the implementation of a service for a group of people from birth to death.
- The frequency with which the patient or a specific group requires care, maybe stratifying by age or risk factors.

WHAT IS THE OUTCOME?

First, we must identify all the consequences of the intervention evaluated.

Next, we must evaluate and decide which consequences are measurable and in what way; then, how the measured consequences are valued, for example in kilograms of weight lost, years of life gained, or quality of life. Not only does this help determine the extent of the outcome but is instrumental for choosing the type of economic evaluation.

1.8. Specifying comparative options

Since time and money invested in evaluations are finite, health services are complex, and intervention effectiveness data are limited, comparator interventions will often have to be chosen from a large number of options. The choice of comparator has a fundamental impact on the type of evaluation to be performed, the collection of the pertinent data and the interpretation of the findings.

The main comparison options are listed in the following table.

| |
|--|
| 1. Standard practice |
| A Single main type of intervention |
| B Combination of interventions |
| 2. Best existing alternative |
| 3. Doing nothing |
| A Without the new intervention |
| B Without any care |
| 4. Alternative intensity levels for the new intervention |

Since decisions about what services to provide are made in the context of what is generally done, the most frequently used and highly relevant comparison turns out to be standard practice. However, it is often difficult to define what the usual practice is since it varies in different areas (for example, in the Argentine Puna cervical cancer screening is not performed while in some centers of the City of Buenos Aires it is performed with a frequency of less than a year). Many authors choose the most widespread alternative as a comparator. The more alternatives are analyzed, the more complex the analysis becomes, and the more information must be collected for this purpose. If we choose to compare all alternatives, we must consider that all patients are eligible for all treatments, which is not always true (for example, a patient with severe cardiovascular comorbidities is not eligible for high-risk surgery).

An important issue to consider is the certain likelihood that standard treatments will not be efficient (such as lung cancer treatment); in that context all comparisons with other alternatives will be efficient. In these instances, you can choose to compare the new intervention to the usual practice or do nothing.

In the table above two options of “doing nothing” are listed: one of them defines not doing anything of what the new intervention proposes, the other implies doing nothing at all. Both alternatives may involve costs and consequences that should not be assumed as null. We see that the first option is more important for current practice, while the second is better for a long-term effectiveness analysis and for considering the health system as a whole.

1.9. Target population

The target population is the group of people to whom the intervention is directed. It can include different ages, sexes, comorbidities, or places of residence, among others. It is important to identify, through subgroup analysis (e.g., age ranges or socioeconomic status) which of them is the most benefitted from the intervention (thus resulting in more cost-effectiveness). This allows efforts to be focused on helping the greatest number of people at the lowest cost, avoiding the inappropriate use of the technology (for example, performing a graded ergonomic test on an asymptomatic young adult for mild physical activity or a mammogram on a young woman with no history).

An important site for disseminating Economic evaluations is the Centre for Reviews and Disseminations at the University of York, which maintains numerous databases and disseminates research results to decision-makers in the British National Health System.

2. Development of economic evaluations

Economic evaluations “mounted” on clinical trials (piggy-back)

An existing methodology for carrying out economic evaluations is the so-called “piggy-back” (embedded in clinical trials). Alongside the execution of trials at any development stage of a technology, effectiveness and safety data are collected along with the consumption of resources. This makes it possible to obtain cost-effectiveness or efficiency estimates from the earliest stages of the long road toward the approval of a new technology, especially in the pharmaceutical field. Quite often, final decisions about the launch of a new product to the market or approval by certain regulatory authorities depend on this economic information as much as on efficacy and safety.

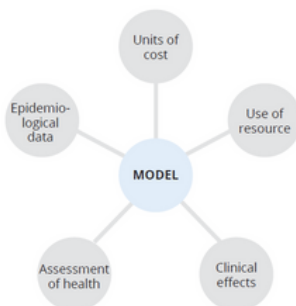
Introduction to Model-Based Economic Evaluations

There are scenarios where it is not feasible to conduct a research study to answer the questions of interest, for example those situations where the outcome is the death of the person or when it must be expected since birth.

On other occasions, data from different sources (for example, administrative databases, medical records, databases of complementary studies) are gathered, so as to analyze them from a new perspective in order to reach conclusions about a specific problem. For these cases, appropriate “mathematical models” are built to enable the collection of information for decision making at the right time.

A “model” is a simplification of the real world, considering only the important components from the perspective to be analyzed. A good model allows us to calculate the odds of events occurring by virtue of the diverse decisions that can be made (answering how likely it is that a certain event will occur). Models help decision analysis and promote an explicit and comprehensive validation considering the existing alternatives. They also facilitate the identification of deficiencies in the current evidence. Models are statistically appealing since they help to show the different probabilities of occurrence of the alternatives, and consequently the hypotheses raised can be tested.

The following figure shows the different types of information that can be used to build a model:



Information needed for model building

Techniques using economic models are becoming increasingly popular to aid in decisions regarding health care behaviors or the introduction of new technologies.

Models can be useful in various instances:

- When a decision must be made in the absence of strong evidence from accessible data
- To extrapolate data beyond the conclusions of clinical studies
- To relate intermediate data to endpoints (e.g., blood pressure and death or bone density and hip fracture)

- To extend the results of a study to other situations (e.g., where the value of medical time or the cost of treatments or the prevalence of disease changes)
- To synthesize a side-to-side comparison of treatments not performed by clinical studies (e.g. treatment of osteoporosis with strontium ranelate and alendronate)
- To indicate the need for further research

A good model should reflect the standard practice and for that purpose it must use a good comparator. It should be based on the best quality information available (meta- analysis, randomized double-blind trials). The entire important period of time must be evaluated so as not to leave out any crucial consequences. For example, the treatment of multiple trauma may last only 1 week, while the treatment of hypertension should be sustained for more than 10 years.

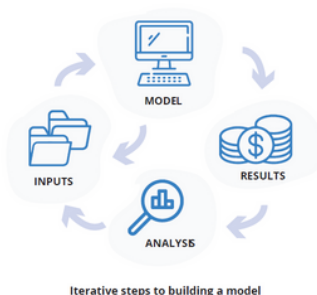
One critical feature models should have is transparency and reproducibility so that they can be adapted to different scenarios. It is essential that a model has internal validity. An ever increasing number of governments (England, Australia, Portugal) are now requiring economic evaluations before approving a new technology, even more so to grant global coverage.

In summary, the main characteristics of “models” for Economic evaluations are:

- They are a means to represent the complexity of the real world in a simpler and more comprehensible way.
- They are mathematical structures that represent the clinical and economic outcomes of alternative strategies in patients or populations in different scenarios.
- They synthesize data from multiple sources.
- They account for uncertainty.
- They focus on the decision to be made.
- They are increasingly accepted as economic evaluation instruments by regulatory agencies

A good model tries to resemble reality faithfully enough to be used as a tool.

Very often, we do not obtain all the data we need to make a decision from a single source of information (such as a randomized controlled clinical trial). That is why it is necessary to resort to “modeling” this clinical-economic problem.

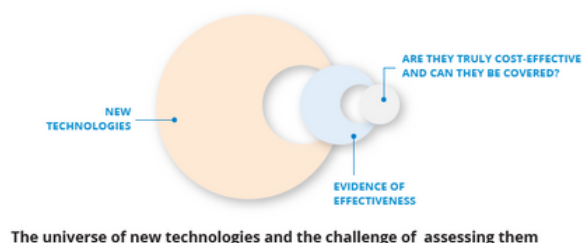


Building a model implies somehow simplifying the clinical-economic problem in some mathematical structure and is usually done on a computer, either through spreadsheets or specific programs. Once the structure of the model has been outlined, the next step is to incorporate the required parameters (epidemiological, effectiveness, costs, utilities) into the model using the best available evidence.

Why are model-based economic evaluations the most commonly used?

Decisions will inevitably have to be made. Even if we take the “do not innovate” stance and continue to treat this health problem with a certain therapy, we are making an “active” decision not to innovate, thus leading to clinical and economic consequences. There are different ways to make decisions:

No country or health system in the world can provide its population with all the technologies that have proven effective. That is why, although effectiveness is a “sine qua non” condition for deciding to adopt new technologies, it is not sufficient per se, and among all the technologies shown to be effective, it is essential to decide which ones should be financed based on the extent of the benefit obtained and its cost. Given the abundance of new technologies, and their significant influence on health cost increases, it is important to “separate the chaff from the wheat” when making healthcare decisions.



The first step in this process is to identify, among all technologies, and especially the new ones, which ones have really proven to be more effective than the current ones. This first decision is mainly based on a thorough literature review and strict evidence-based medical criteria.

Most new technologies do not usually represent a clinically significant improvement for patients; so in a second step, it is now necessary to determine, among the effective technologies, which ones are cost-effective, or denote a good use of the system resources.

What does it take to make decisions? What can a model afford in this regard?

In order to make an adequate decision about a possible technology, the main questions that must be answered are the following:

1. What are the tangible benefits afforded by the treatment compared to the best alternative? And compared to others? In general, it should be possible to determine benefits on account of:
 - Clinical events
 - Survival
 - Quality of Life
2. What are the costs?
3. Then, what is the incremental cost-effectiveness of the treatment?
 - In the study population
 - In the real world and in different groups
4. What is the impact on health budgets?

There is an acknowledged consensus that models are more useful to decision makers than clinical trials. This is because virtually no RCT provides many aspects that are relevant to decision-making. Among them we highlight:

- Comparators
 - I need to make the decision using all the relevant comparators for a certain health problem, not just the ones usually assessed in Clinical Trials (new drug versus placebo or usual treatment)
- Times
 - Real-world data is generally not available at the time of decision-making, and even if it is available, there are seldom long-term consequence data.
- Costs and Efficiency
 - Outcome modelling is generally easier, faster, and less expensive than collecting large-scale health and cost data.
- Flexibility
 - A wide variety of scenarios and strategies can be tested to identify the most efficient and equitable resource allocation.
- Extrapolation
 - To other countries the regions.
 - To other populations.

This does not detract from the usefulness of Clinical Trials, but the message is that Economic Evaluations based on Clinical Trials alone are very rarely sufficient to make a proper decision.

The following graph shows examples of sources of information to incorporate the different parameters of interest into the models.

| Type of parameter | Source |
|----------------------------------|--------------------------------|
| Baseline event rate | Observational Studies / RCTs |
| Relative effect of the treatment | RCTs |
| Long-term prognosis | Longitudinal Obs. Studies |
| Use of resources | Observational studies / RCTs |
| Quality of Life (utilities) | Cross-sectional studies / RCTs |

Summary

The different steps to performing an economic evaluation are:

- Definition of the health problem;
- Build a model that allows to conceptualize the health model;
- Populate the model with the different data required (disease burden, effectiveness of interventions, quality of life, costs, etc.);
- Estimate the cost-effectiveness rate of the intervention to be evaluated in relation to its comparators;
- Perform the uncertainty analysis;
- Interpret the results obtained and put them into context

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