

Chapter 2

Logic and Methods of Evaluation in Healthcare

Abstract Starting in first chapter with the study of international management on the evolution of PA the present chapter aims to study the evolution of decisional logic that has affected the NHS the analysis of the approach used in support of decision better known as Health technology assessment (HTA). Therefore, in the first part, this chapter describes how the different approaches, succeeding one another over time, in the study of public administration, have influenced the evolution of logic and the tools used to support public decision. This introductory part presents the theoretical framework of reference, on which the sample model used to illustrate how the evolution of management theories has influenced the decision-making processes related to the selection of the different allocative solutions to be applied to the healthcare systems, on which it is based.

Keywords Economic analysis • Planning • Evaluation • Cost-benefit analysis • Cost-effectiveness analysis • Cost-utility analysis • Cost minimization analysis

2.1 Evaluation Issues in Health

The NHS is a system based on two main elements: Planning¹ and Evaluation.² This statement of principle, even though inferable by all the regulations that in the last thirty years have governed the entire NHS system, has actually found greater

¹ In a healthcare company, the processes of planning and operational control must, on the one hand, contemplate the elements of predefinition of resources to be used for the pursuit of the assistance objectives that they set out to achieve, and on the other, to foresee activities of verification for the results obtained, in order to correct deviations and reshape the system of benefits and services. In particular, the programme, must conduct a careful examination of the resources it has available in terms, above all, of professional skills and technological resources in order to achieve a fair and proper allocation of such integration, which aims to achieve the predefined objectives according to the daily complexity of assistance and expectations of users. Programming and health organization manual.

² The handling of economic evaluation in healthcare specifically aims to influence this level by drawing attention to some of the tools of economic analysis which facilitate substantial improvement in the quality of decisions (Meneguzzo 1995).

application in terms of programming rather than evaluation. However, it is clear how the two functions must be closely interrelated, since programme orientation cannot but result from analysis and assessment of the needs and organization of supply. In fact, the evolution of the standards and State-Region agreements in all these years have taken into consideration primarily, if not exclusively, evaluation intended as an analysis of work to ascertain the conditions of economic equilibrium. In other words, so far, a general definition has been privileged that has given supremacy to procedural-administrative assessment which foresees a in depth inspection of healthcare management performance and on spending, many: vice versa, little has been registered in terms of treatment, efficiency and performance outcomes of the system, verification of system operation criticality, both nationally and regionally. On the other hand, it should be noted that when processes of measurement testing were activated, it was an experiences characterized by poor circulation of information and little, if any, intensive sharing of tools and methods used. In considering the experiences, however, we must recognize that the difficulty to activate the widespread evaluation processes is understandable, since the evaluation function is closely linked to that of inspection, and on this issue there have been major conflicts between the two levels of institutional government of the system: State and Region.

To change this state of affairs, the Health Pact—signed on 28 September 2006—with the logic of promoting a common set course between the government and the regions, with the Regions introducing the concept of “*self-assessment*”. The quality of healthcare provided, indicating the need for “*the central level (in both ministerial and inter-region coordination) not only for the function of inspection but for the regions that require it or at least for those engaged in recovery plans, service and support aimed at self-assessment of the quality of healthcare provided.*”

In the Health Pact, an innovative national role is affirmed for the Regions by way of strong coordination and subsidiary support of reality with great criticality.

Assessment, on the other hand, assumes in this sense techno-scientific connotations only in part, a field in which possible conflicts are always solvable, once the mutual regional and inter-technical system of “mistrust has been overcome”. In this case, evaluation becomes, the field in which, more than any other, technique and politics meet; an evaluation process detached from the political context does not exist and it is not such if it does not foresee its fulfillment the activation of a decision and, if the assessment is aimed at decision-making, it will be the latter to control the content.

From this point of view, evaluation can also appear awkward, making us wonder if evaluation is really the aim of the system. The answer to this question is not simple: institutional decision-makers carry out their roles mainly as a result of evaluation processes, only that very often these processes are informal in nature and are based on political relations with subjects in need and the professional world.

The limit of this approach has gradually become clear as too high is the risk of favoring those stronger or more represented by the system and does not focus on the real needs of the population and the organization of services. It is precisely for this reason that, from the perspective of an ex-ante evaluation, a very refined new tool is becoming more and more widespread that allows “technicians” to provide

“decision makers” with useful information in order to choose the best possible alternatives. There is talk of HTA and specifically, in this chapter, the focus will be on economic evaluation.

2.2 The Role of Economic Analysis in Healthcare Processes

Economic organizations and all of the productive sectors must address three fundamental economic issues: “what to produce”, “how to produce”, “for whom to produce.”³ In the healthcare sector, the “what to produce” means to choose among a great amount of services including the healthcare ones, provided by medical and technological evolution, that actually provide benefits for citizens; “how to produce” means to select the most suitable manufacturing process for the production and provision of healthcare services (for example to perform minor surgery in the form of short hospitalization, *day surgery* or outpatient), “for whom to produce” means to decide which citizens have the right to healthcare and how (all free of charge or with a sort of cost-sharing on the basis of per capita income).

In almost all the productive sectors, the three fundamental economic issues mentioned above are solved via the free market through the mechanisms of supply and demand. In healthcare this is not possible, because the healthcare market presents some peculiarities, for example, the consumer or customer does not really know what he needs and this is why a doctor is addressed, who decides the most suitable diagnostic therapy; in healthcare the consumer-customer does not directly bear the cost of its consumption due to the presence of the so-called third-party payer (State or insurance). The alternative free market tool is programming: the State determines what to produce, how to produce it and for who to produce it through a planning process, which makes use of the appropriate means of evaluation, such as the economic valuation techniques. In an ideal world characterized by the limitlessness of resources available in Healthcare, assessment of the effectiveness of healthcare interventions would represent the only decision criterion in making choices relating to prevention, diagnosis and treatment of diseases. However, in reality, the limited economic resources involve the introduction of the “economic rationality” concept in healthcare choices and the definition of criteria that sustains the process of allocation of resources so that they are not used in an inefficient manner.

The assumption of economic rationality lies in the fact that, exactly because they are limited, economic resources have a cost (cost-opportunity)⁴ represented by the benefits obtainable with the same resources at the moment they are used in an alternative way. In other words, seen that resources are scarce, the decision to

³ The production of any good or service, including the production of healthcare goods, can only occur through the use, consumption, and the application of certain resources. The resources that allow the production of a good are called inputs: therefore a production process consists in the transformation of resources in product. The production function describes the technically efficient methods to transform inputs into outputs.

⁴ The cost of a unit of resource is the benefit that would be derived from its best alternative use.

finance a specific healthcare programme, automatically means, to decide not to fund an alternative programme, and the cost of the choice made is represented by the sacrifice imposed by the discarded best alternative.

Therefore, economic evaluation in healthcare, understood as the set of logical and methodological tools aimed at dealing with the problem, based on the principles of economic rationality, of choosing which alternative resource to use, mainly aims at sustaining healthcare decision making to avoid making choices that are carried out randomly or exclusively on the basis of political or ethical criteria when trying to solve the problem.

2.3 The Main Techniques of Economic Evaluation in Healthcare

Analyzing the healthcare system, economic evaluation⁵ turns out to be a mandatory component for a rational approach that conforms, on the one hand the necessity to respond to the growing needs for public assistance and on the other the lack of resource which is needed for the fulfillment of all healthcare necessities. The task of healthcare economy is precisely to sustain the various decisions, in order to provide decision makers with useful information to make targeted choices; a choice cannot be entrusted to randomness, even in the consideration that, once used, resources are no longer available.

Healthcare economy is the study of the ways in which individuals, organizations and companies accomplish choices regarding the allocation of resources to respond to the public healthcare needs (Drummond 2000).

The healthcare needs have been defined by the reforms introduced over years, giving Regions the responsibility for the management of resources. Consequently, healthcare companies have been equipped with organizational autonomy, especially with the intervention of healthcare experts in assessing activity programmes to be carried out rather than the innovations to be introduced (*devices*, drugs, etc.).

Healthcare experts, in their course of duty, are increasingly called to respond to economic issues, due to the responsibility they have in the use of resources and the achievement of the “healthcare” *mission* (Jefferson et al. 1998).

In the context of healthcare economy a distinction must be made, between healthcare economy, understood as a field of study, and healthcare economy, understood as a method of thinking:

⁵ There are several definitions of the term “economic evaluation”:

- “Essentially, the cost-benefit analysis consists of a comparison of the costs and benefits of a given number of programs that are alternative or competing”;
- “The economic evaluation is a comparative analysis of the costs and consequences of alternative strategies of action” (Drummond et al. 2000);
- “The cost-effectiveness analysis is a method aimed at assessing, in a comparative sense, the impact of the allocation of expenditure on various healthcare interventions”.

- *Healthcare economy* understood as a field of study involves researchers formulating new ideas and the search for typical structural relationships, which give rise to a set of conceptual tools and systemic analysis.
- *Economy* understood as method of thought, or discipline, is instead a way of approaching issues and non economic human activities, using conceptual tools and analysis to deal with the problems of the sector in a systemic manner.
- *Healthcare economy* understood as an area of study, which takes into account some essential considerations such as the definition of the objectives of healthcare activity, improvement of treatment and management of resources.

With regards to the first two aspects, it should be noted that healthcare economy is a science that, when made available to healthcare experts, tries to optimize the use of resources based on the achievement of healthcare goals as effectively and efficiently as possible.

As for the last aspect, it must be considered that today, the demand for resource is oversized compared to the actual demand, in the sense that the destined user may make a request even when there is no real need. For this reason, attempt should be made to optimize the use of resources through careful management of the same. Healthcare economy is a discipline that deals with the problems related to the effort put into choosing, various alternatives, comparing them and enabling healthcare providers to make conscious decisions.

Using economic methodologies allows you to observe the transparency and complexity of healthcare activity, by relating the use of resources to the achievement of final results (outcome).⁶

The economic perspective focuses on the amount of resources used by a service and the quantifiable consequences. However, it is simplistic to consider that economic evaluations in healthcare only aim at costs because, if so, the maximum efficiency⁷ would coincide with doing nothing, which in fact has a cost approximately to zero. Furthermore, to define the economic perspective only in terms of cost minimization means neglecting the most important principle, namely the one according to which the resources absorbed by a performance are no longer available for other uses.

A very important issue in terms of economic evaluation concerning the clarification of all costs (in this particular context, cost indicates the economic burden of

⁶ Please refer to Innovating innovation. See: Liguori et al. (2009).

⁷ There are two different types of efficiency: *allocation efficiency* evaluates various alternative interventions to decide how to distribute resources between various interventions in order to obtain the maximum benefit. *Technical efficiency* evaluates, the best way to achieve a certain goal (Jefferson 1998).

illness) and benefits according to the category “direct/indirect”, “tangible/intangible”.⁸

Direct costs are those incurred by the healthcare services, the community and directly by the family, to treat a disease; indirect costs mainly consist of production losses⁹ that rebound on families, individuals and society; intangible costs, are those caused by pain, suffering and loss of time.

The criteria used for the classification of costs are also used for the classification of benefits, in order to have a common means of assessment that can be used in the course of the evaluation.

Achieving reliable outcomes in economic evaluation is the result of logic and the application of criteria which are not always definable. The margins of discretion, of who carries out the study, are faced with limits in the accuracy placed in the definition of objectives, the choice of recruitment, selection of analytical methods and data.

The detailed analysis of the objectives of the study is undoubtedly the starting point of the analysis. If you do not know the exact objective, it will be difficult to establish a strategy or a process. The description of the objectives should be as detailed as possible, including not only quantitative measures, but also the qualitative ones.

The description of the objectives leads to the identification of healthcare related problems and related interventions to be compared: in this second phase, the main acting parties, organizational relationships and the different scenarios to compare are identified.

⁸ The costs and benefits are divided into direct, indirect and intangible:

- Direct costs and benefits: are used to indicate the resources saved and consumed by the programme in respect to an alternative choice. They generally refer to the resources in the healthcare sector, but sometimes also include the costs borne by the patient.
 - Indirect costs and benefits: in the past this expression indicated the time that the patients consumed at their disposal according to the given health program. In the language of elementary accounting they are referred to as overhead costs
- Intangible costs and benefits: in the past, these terms were used to indicate the consequences the difficulties in measurement and evaluation. However, these words are not costs or resources taken away from other uses, and are not even intangible seen that through the measurement of utility and willingness to pay they can be measured and evaluated.

⁹ Productivity losses relate to working time lost caused by disability or premature mortality. Productivity losses are estimated by applying the average incomes by age and sex to the working time lost. Productivity costs for premature mortality consist of labor income expected in the entire working life of an individual, because if the man had not died, he would have continued to be productive until retirement (Tarricone 2004).

The next step is to adopt a point of view and the communication¹⁰ of the same to all the people who will eventually be involved in the analysis. The choice of a point of view orients the analysis and becomes the base on which to build the entire system of evaluation. In this step, the type of analysis and the scope of reference are chosen.

In terms of methodology, the economic evaluation aims at aiding decision-making in the use of resources, both in consideration of possible alternative uses, and in consideration of the scarcity of resources principle. The variables that are taken into consideration are the number of alternatives, the depth and understanding of economic evaluations.

From a methodological point of view, economic evaluations produce results as much appreciable as comprehensive. All methods of economic evaluation are attributable to the same principle: the analysis of one or more healthcare intervention comparing the inputs (resources for the implementation of interventions) with the output (consequences, effects of the intervention).

To evaluate the input and the consequences of each action, all methods of economic evaluation use the same procedure in three stages:

- identification of all the inputs and outputs of each action;
- assessment of both with appropriate units of measurement;
- determination of the economic value of resources and the consequences.

The distinctive feature of an economic analysis is the choice of alternatives, as the lack of limited resources and the consequent inability to produce what is desirable, makes it essential to make choices in all fields according to certain explicit and implicit criteria, trying always to facilitate the choice of allocation of scarce resources. Assigning economic value to the resources and the consequences is, certainly, the most complex aspect of economic evaluation because it attributes an economic value to the outcome of “*healthcare*” it is not a simple process, at least not for the ethical implications.

The term “*economic evaluation*” refers to a comparative analysis, both in terms of cost and consequences, including alternative ways of action. This means that the main functions of an economic evaluation consist in the measuring, identification, development and comparison of the costs and consequences of alternatives considered (Table 2.1).

Comparing the results makes it possible to organize the healthcare programmes based on the ratio between the costs and consequences making choices on the basis

¹⁰ In this sense a distinction between information and knowledge is also fundamental, because, knowledge is information that changes something into someone, both becoming cause of action, and making an individual (or organization) capable of different or more effective actions. If the information is constituted only by a set of data entered.

Table 2.1 Classification of economic evaluation

No Only the consequences are examined		Yes	
Only the costs are examined			
No	<i>Partial evaluation</i> 1A Description of the results produced	<i>Partial evaluation</i> 1B Description of costs	<i>Partial evaluation</i> 2 Descriptions of costs/results produced
Yes	<i>Partial evaluation</i> 3A Effectiveness evaluation	<i>Partial evaluation</i> 3B Cost analysis	<i>Completed economic evaluation</i> 4 Cost minimization Cost-efficiency analysis Cost-utility analysis Cost benefit analysis

Source Drummond et al. 1987

of the budget.¹¹ According to Drummond, one of the fathers of economic evaluations, techniques (of evaluation) can be classified as partial and complete depending on whether two or more alternatives are compared and the consequences of the options considered. The absence of even one of these elements characterizes the partial nature of the analysis.

Are the costs and consequences of each alternative examined? Are two or more alternatives compared?

Quadrant 1: we simply describe the results of a programme or its costs without comparing alternative programmes.

Quadrant 2: even in the absence of alternatives, the description given considers both the costs and the results (in some cases it refers to cost-consequences).

¹¹ The budget is therefore a comprehensive programme in the sense that it tends to consider the company’s overall economic and financial equilibrium as the result of a coordinated action of the various components of the organization, in the drafting of which the fundamental elements pre-listed must be taken into account:

The budget therefore has a plurality of purposes, which are not only reduced to the financial aspects. These include instances of:

1. *Programming*: the objectives are decided with the budget, the resources are allocated in a consistent manner, and the achievement of the same is verified.
2. *Guidance and motivation*: guidance, because it provides executives with a document indicating which goals to achieve and by what means; motivation by assigning targets to the CdR
3. *Monitoring and evaluation*: it provides the parameters against which to compare operating results actually obtained.
4. *Coordination and integration*: as it ensures that the goals and decisions of individual CdR do not conflict with each other and are consistent with the overall objectives of the company.
5. *Learning and training*: managers need to take on a more “managerial” approach.

Quadrant 3: different programmes are compared, considering, however, only the costs or the consequences (both aspects are not taken into account). In the event that only the costs of alternative programmes are taken into account, it refers to cost evaluation, if you take into consideration the consequences it refers to evaluation of effectiveness.

Quadrant 4: Assessment is complete because multiple programmes are compared and in each one costs and consequences are identified.

The full economic evaluations are, as has been said, those that compare two or more alternatives by relating the costs and the effects they have on the health of patients. The main ones are:

- cost-benefit analysis (CBA)
- cost-effectiveness analysis (ACE)
- cost-utility analysis (ACU)

The evaluation techniques listed above, are similar in terms of cost estimation, it tends to provide a value of the cost elements expressed in monetary units.

The differences, however, emerge from the evaluation of the effects: in CBA effects are measured in monetary terms i.e. with units same as those used for the costs; in ACE the effectiveness is measured in physical units i.e. in years of life earned; in ACU the effects evaluated may be different.

The summary of analysis that can be inferred from Table 2.2 summarizes as the concepts of:

- **cost minimization analysis (CMA)** is applied to determine which treatment is most economical of a number of options having the same efficacy and similar therapeutic outcomes;
- **cost-benefit analysis (CBA)** is used to calculate the connection between costs and clinical benefits (both expressed in monetary values) is performed in relation to a single treatment or in the comparison of several treatments that have the same therapeutic purposes, but different clinical efficacy;
- **cost-effectiveness analysis (CEA)** is used to calculate the relationship between costs and benefits (the benefit expressed in physical units or clinics) it is carried out in relation to a single treatment or to compare various treatments for the same therapeutic purposes, but with different clinical effectiveness;
- **cost-utility analysis (CUA)** is applied to calculate the ratio between costs and benefits monetizing the cost and expressing the benefit in clinical units that incorporate an estimate of the quality of life of patients (so-called *quality-adjusted life years* or QALYs).

In economic evaluations, the resources consumed in choosing a programme (or technology) must be exploited in order to represent the opportunity cost, i.e. the value that the resources they would have had if they had been used in the best possible alternative, in other words, the value of the benefits which should be given up applying the resources in an alternative programme.

Table 2.2 Techniques of economical evaluation

Type of analysis	Measurement/evaluation of costs in both alternatives	Identification of effects	Measurement/evaluation of effects
ACE	Monetary units	Only objective-result common to all the alternatives obtained at different level	Units of physical measure (number of years earned, days of illness avoided, etc.)
ACB	Monetary units	One or more effects not necessarily common to both the alternatives and obtained at different level	Monetary units
ACU	Monetary units	One or more effects not necessarily common to both the alternatives and obtained at different level	Days of wellbeing or QALYs
Minimization of cost analysis	Monetary units	Identical in all the relevant aspects	None

Source Drummond et al. [1987](#)

In fact, both the costs and the consequences are distributed in various ways in time and as a consequence will only make sense if they refer to the same period of time: therefore, it takes mathematical methods to update them.

In order to introduce, the techniques of evaluation in detail, it is appropriate and necessary to dwell on some of the concepts already set out in the tables:

- **Monetary type unit** is used in the presence of a target market both for the costs and the benefits to assess. The treatments according to the various tariffs, the cost of nursing care, and the cost of pharmaceuticals are all examples.
- **Indicators expressed in physical terms** are examples of the number of hospital infections, hospital mortality, days of hospitalization, and the values of body temperature.
- **Indicators of health value** are summary indicators of the *outcomes* related to the functional status of the patient, to the satisfaction of the need for care and generally related to the notion of the concept of health.
- **Indicators of utility**, expressed in terms of life years gained valued with the quality of life generated by the interest concerning it (QALYs).

For the purposes of economic evaluation, the evaluation of the organizational criticality issues acquires particular importance because the choice of an alternative can have direct effects on organization and indirect effects on the budget.

The application of a scientific method for the economic evaluation in healthcare articulates from a series of questions:

1. What are the benefits to be obtained?
2. How to quantify them?
3. What are the relative costs of alternatives (which methodology to apply)?
4. What alternatives or combinations of alternatives are the most convenient?
5. Once you have chosen the alternative, which is the best way to apply it (optimize)?

The conceptual references at the base of economic evaluation are multiple and must be taken into complete consideration, not only for the completeness of the analysis, but also to allow the application of the study following different points of view.

The following paragraphs will summarize, the main valuation techniques, emphasizing the limits and potential of each.

2.3.1 Analysis of Cost Minimization

As already mentioned, one of the main problems of public and private healthcare systems is finding and allocating resources. In the coming decades, the phenomenon will be increasingly important for healthcare decision makers given the continued aging of the population, the increase in life expectancy and technological progress.

It can be inferred from what has been written that the basic concept of economic evaluation¹² is “cost opportunity”, that is, the benefit we have renounced to in using the available resources in the best alternative. After having analyzed the aspects of technical evaluations, starting from the partial ones and concluding with the complete ones, we must reiterate that economic evaluations have limits, the most important of which are:

- poor methodological quality
- conflicts of interest (efficacy results of new drugs and funding of studies);
- socio-economic differences of the population served, varying the appropriateness of the services.

In the practice of economic evaluation there are still two delicate points to highlight: the choice of the alternative with which to compare and efficacy data under study. The quality of efficacy data that concerns assessments should be

¹² Drummond has examined a number of examples where economic evaluations are used to sustain some decisions. In general, however, the use of economic evaluations is still limited in relation to its potential. There is a number of reasons underlying this limited use, including the lack of dissemination of the results, the decision makers’ lack of credibility towards their importance, the lack of understanding of the results and the absence of mechanisms for using economic considerations in decision making.

based on a controlled clinical trial or a methodical review.¹³ In the event that there are no experiments, it is necessary to refer exclusively to the model that shows the limits highlighted first.

The last issue, not in order of importance, is to decide which costs should be included in economic evaluations. Usually, it takes into account only the direct costs if the point of view is the one adopted by the NHS, and will take account of those costs if the intangible point of view is that of patients and societies. In the case of diseases with a significant amount of indirect costs (migraine, multiple sclerosis...) not to adopt the general point of view of the company (also considering the indirect costs) can lead to very different conclusions in respect to not considering them in the analysis.

In order to assess the sturdiness of the economic evaluation results, a sensitivity analysis¹⁴ must be carried out, a process through which all costs and benefits are varied. It will result in a univariate analysis if it is only one parameter that varies and multivariate if it is more than one parameter to vary. In this last case it will be possible to evaluate the best case (lower costs for a more effective new alternative) and the worst case (greater costs and worse effectiveness).

Regarding the results, economic evaluation when comparing innovative treatment A and old treatment B, may result in the following situations:

Cost minimization analysis (CMA) is among the partial evaluation techniques and is intended as an economic analysis that is applied to determine which treatment is the most economical out of the various options that have the same efficacy and similar therapeutic purposes.

¹³ Systematic reviews provide a means to efficiently summarize the information on which to base clinical decisions. They have the objective of making the reader aware about the rapid identification on how research is progressing on an intervention, the evaluation of the methods used in primary studies and the extent of the effect of a treatment in different contexts. In order to decide if there is enough evidence on the effectiveness of an intervention or if it is necessary to conduct further studies to evaluate a treatment and what aspects should be considered. Methodical reviews differ from traditional narrative reviews of literature. The later require a great deal of influence in selecting the author to study, critical evaluation of the studies and the synthesis of the results. Methodical reviews vice versa follow standard protocols whose basic elements are the completeness of the research studies, assessment of the quality of the studies to be included and the ability to quantitatively synthesize the results through meta-analysis (Dictionary of the New Healthcare).

¹⁴ These are calculation methods to analyze if changes, in some of the main variables, cause variations in the analysis results in the field of economic analysis or *decision-making*. Suppose, for example, that a system of community assistance is set up to prevent unnecessary hospitalization. It is achievable only if you have home nursing units able to assess the patient's condition and to ensure that the service, which they actually need, is provided for them. However, it is not clear what would have happened to the patient had it not been able to use this type of service. We can therefore try to establish to what extent it is possible to achieved a saving, avoiding unnecessary hospital admissions, changing the parameters related to:

- cost of the patient hospital stay in the event of a admission (cost);
- number of patients hospitalized in the absence of a service (volume).

This analysis identifies which is the most efficient, that is the one that makes best use of available resources, focusing only on the input without comparing the consequences.

CMA assessments are based on the description of the costs and aim to identify the less expensive alternative with reference to the solutions with identical *outcomes* or with less significant differences. The CMA does not merely describe the costs, but verifies the equivalence of the alternative *outcomes*, also in consideration of the scientific evidence reported in studies (Jefferson and De Micheli. Economic interventions of healthcare evaluation. Scientific thinking publisher).

CMA examines the possibility of reducing costs while keeping the outcomes of the various alternatives stable and stimulating healthcare procedure innovation, healthcare activities and relationship with the environment. The peculiarity of CMA is that of focusing its attention on the monetary costs, comparing the different alternatives that produce similar effects on the patient's need for care.

CMA provides lists of costs and consequences of the different alternatives. The analysis is all the more impressive when the cost items and benefits included are documented with scientific reference and productive characteristics of company activity.

The most significant CMA limit arose from the need to compare the alternatives that produce similar outcomes: it must be considered that over the years the various authors who have dealt with the subject of economic evaluation have never explained what it means, to get the "same effectiveness"¹⁵ or, conversely, the "different effectiveness" especially in light of the uncertainty that surrounds the sample data available to the researcher.

2.3.2 *Cost-Benefit Analysis*

It is an analysis in which both the input and the consequences of various healthcare interventions are expressed in monetary terms so that they can be directly compared to alternative interventions.¹⁶

CBA is a full economic evaluation technique in which the assessment of costs is made in monetary terms. This methodology began to take on an important role in

¹⁵ Effectiveness in healthcare means more than anything else the success of the medical service and consequently the complete satisfaction and well-being of the patient. It is customary to make a difference between efficacy, that is, the ability to achieve the desired results and effectiveness (concept used in healthcare) the ability to achieve the expected results under real conditions in the given time. "Healthcare: how to act effectively and efficiently." Project Noyag. Edited by Domenico De Felice and Luca De Felice.

¹⁶ William was one of the first to use the term cost-benefit approach in 1974, meaning it as a simple description of the economic approach in the healthcare market, whose main problem is expressed in terms of relationship between resource use and healthcare services production.

the last century in the United States, following the diffused idea that it was necessary to develop social justification for projects funded with public money.¹⁷

Very often decisions must be made on how to use the economic resources for costly interventions that produce expenses and consequences that affect the entire society.¹⁸ There are situations where healthcare interventions are in competition with alternative interventions that may or may not have anything to do with healthcare, basically, CBA aims to compare the costs and the social consequences of different interventions or compare them to the alternative of not implementing any intervention. The basic assumption of CBA is in the belief that social welfare can be increased by allocating additional resources to those sectors from which the greatest marginal benefit may derive.

CBA analysis poses a question to whether it is worthwhile to accomplish a given intervention or not. CBA activity is the listing of all the costs and consequences of a particular action which is necessary whenever it comes to goods or services, such as healthcare. However, to transform healthcare consequences into monetary terms is difficult and it is these difficulties that have resulted in CBA losing its popularity.

The CBA indicator is the ratio between the total sum of the benefits and costs attributable to programme-healthcare intervention. The results should show the costs and benefits excluded from the analysis as well as the methodologies used.

CBA does not find wide application in healthcare due to its difficulty to assess, in monetary terms, the benefits and costs associated to the evaluation of a healthcare programme. However, various approaches have been used; particular reference is made to the theory of individual preferences or the social and human capital theory (Jefferson et al. 1997).

The methods for preference assessment can be explicit and implicit. The method of preference expressed assesses the individual's willingness to pay based on consumers decisions: the choices are real and not hypothetical. The contingent valuation approach¹⁹ is to directly ask individuals if they are prepared to pay (willingness to-pay²⁰) or to accept (willingness to accept) to get the benefits of a specific healthcare programme which is described in terms of hypothetical scenario.

¹⁷ In the case of public projects the issues to be considered are the eligibility of the project (social relevance) and the type of loss (economic sustainability).

¹⁸ The studies on the social costs of disease, measure the economic resources absorbed caused by the existence of a given disease. These studies say "how much do" patients with Alzheimer's disease or multiple sclerosis cost. But what is the meaning of putting, the adjective "social" and the word costs, side by side? It means to measure the cost from the perspective of society.

¹⁹ It characterizes the evaluation of the technical contingency willingness to pay (WTP) and willingness to accept (WTA). The WTP is based on a sample of the general public who are asked how much they would be willing to spend to get a certain benefit or to avoid a certain problem. The WTA, conversely, determines which is the minimum amount that a person would accept to compensate for the loss or reduction of a certain good or service (Drummond et al. 2000).

²⁰ Authors have suggested that determining the shadow prices for a QALY can be a bridge between ACU and ACB.

Compared to the method of expressed preferences, which focuses on the monetary valuation of health risks, with the contingent evaluation method all three categories analyzed (health, not health and external) can be measured.

The human capital approach is based on the calculation of individual social value based on its present and future ability of earning. Each person is a productive resource for society: diseases obviously reduce this production ability and the value of this reduction corresponds to the loss of individual utility.

The adoption of CBA, from a corporate point of view, means that all those costs that are transferred from one company sector to another are not taken into account.

2.3.3 Cost-Effectiveness Analysis

It is an analysis of full economic evaluation that aims to calculate the value of resources used per unit of output, making a comparison between costs (expressive of the use of resources) and effectiveness (expressive of result dimension).

CEA²¹ foresees the possibility to assess the cost per unit of effectiveness for each alternative; the preferred alternatives, with equal effectiveness, are the ones with the least cost or, with the same cost and those with the maximum flow of efficacy. The logical context in which CEA is used is one in which the decision to intervene on a given problem has already been made and the evaluation study is carried out to identify the most efficient way to achieve a certain goal.

CEA is basically applied in two cases:

1. Allocate a budget by choosing a number of alternative programmes, with the objective of maximizing the benefits expressed in units of effectiveness;
2. Achieve a level of objective efficiency, by sustaining the lowest cost.

CEA evaluates technical efficiency in the sense that for each intervention the cost of natural result per unit is evaluated so you can choose the alternative that, for the same total cost, allows the maximization the effectiveness of the choice.

In order to apply CEA correctly, data available in reference literature, opinion of experts and the prospective studies must be referred to.²²

The alternatives that characterize a CEA analysis can be represented by a tree of decision that not only describes the choices, but also the effects and the costs of individual alternatives.

²¹ Reference literature is full of examples of cost-effectiveness analysis, e.g. there is an example of cost-effectiveness estimation analysis to compare activities that do not directly produce effects on healthcare, but pursue other clinical purposes that can clearly be associated with improvements in patient health. It is interesting the comparison of different types to diagnose venous thrombosis in terms of cost per case diagnosed. Along the same lines, two treatment programmes for hypertension are also compared, one at the place of work, the other at the general practitioner's clinic, in terms of cost to decrease the mm of Hg dialostic pressure in the blood.

²² The main source of efficacy data is available in medical literature. The use of this data poses two problems: quality and relevance.

The greatest CEA limit is that it does not take into account the costs and the indirect effects of various alternatives, so this analysis cannot be extended to the whole community without having first considered the indirect and intangible costs and effects.

Furthermore, CEA suggests that the results of the different alternatives be modified only in quantitative terms (use of resources, years of survival), but does not take into account that variations could also be qualitative (Jefferson et al. 1998).

2.3.4 Cost-Utility Analysis

CUA is designed to overcome the limits of CEA, to analyze the results of the possible healthcare interventions that refer to the quality in terms of health gained or health problems avoided. The CUA has many resemblances with CEA, but while the latter uses physical units to express the results, the CUA results are expressed in terms of usefulness.

From the healthcare point of view, utility²³ indicates the state of well-being that the individual is able to obtain from the use of a healthcare service.

CUA is used at the moment in which the choice of a particular intervention must be made, not only in function of quantitative, but also that of qualitative (utility assessment). Other cases in which CUA is preferred are those that give importance to the evaluation of the discomfort of an intervention, when its benefits are felt long after, using a common evaluation of qualitative (e.g. Improving cancer patients' quality of life over a longer period compared to its reduction over a shorter period, or the dialysis patients' quality of life compared to kidney transplant).

Finally, CUA is used to compare healthcare programmes with very different effects, so it is necessary to identify the common unit of assessment when comparing.

Very often the terms usefulness, value and preference are used interchangeably, but in actual fact they are not. The preferences²⁴ contain all the evaluating methods that foresee an assessment of the same through expression of preference of different phases and relative levels of the quality of alternative life.²⁵ According to the technical ability of detecting preference, utility preference is expressed in conditions of uncertainty or risk (the technique is the *standard gamble*) and value

²³ The term utility has been used for some centuries now, is used in various disciplines, and has a number of similar meanings, similar but different.

²⁴ The preferences assessed can be ordinary or cardinal. For ordinal preferences you just have to reorder the outcomes according to a certain degree, for the cardinal preferences it is necessary to connect the outcomes to a number that in some way represents the power behind a decision for that outcome in respect to others. See Jefferson et al. (1997).

²⁵ If a decision maker followed the meaning of the motto *divide et impera*, as a strategy of his own decisions, he should rely solely on the utility theory given that health almost always involves uncertainty. What's more its decisions would apply to the individual and not for the community.

when the detecting technique is carried out confidently (the technique can be the rating scale or time *trade off*).

A solution to solve the problem of usefulness proposed by economists is the use of *QALY* that expresses years of life in respect to the preferences that individuals associate to a year of life lived in a particular health condition.

The preferences are expressed on continuous scales ranging from zero (death) to one (complete well-being) and, as mentioned, are recorded on the basis of standard methodologies.

Rating scale: this detection method foresees that the interviewee gives the health *outcomes* an order of preference from the most preferred to the least preferred, and shows the output on a scale so that the intervals represent the difference in perceived preferences.

Time trade off²⁶: this tool developed by Torrance, contemplates the choice between two alternatives: finding oneself in condition A for a certain period of time equals T; finding oneself in a condition of good health for a given time less than “A” followed by death. The duration varies until a given person becomes indifferent to the two alternatives.

Standard gamble²⁷: is the most consistent method of usefulness. In this approach, individuals must choose if to live their lives in the current health status or bet on a risk. The probability of winning the bet is varied until the given person becomes indifferent to the maintaining of his state of health, or accepts the challenge: this point of equilibrium is the utility that the given person attributes to the bet, and so, to his state of health.

Regardless of the methods used these preferences assess the results of healthcare interventions by comparing them to the quality of life.

CUA evaluations that use *QALYs*²⁸ can compare the obtainable number from an alternative use of resources, or the costs necessary to achieve a given number of *QALYs*.

QALY intends to use, the quantitative and the qualitative dimension of life expectancy in a single numerical expression. Economic evaluation of healthcare interventions.

The usefulness, in this particular case, is the value of the probability of being in a state of health to which the individual is indifferent. The value attributed to the state of health is the value (ranging from 0 to 1) that tries to “make amends”, that is to say, assess a patient’s life expectancy affected by the disease studied to generate *QALY* reference.

²⁶ In *time trade off* the preference are assessed in relation to each other by means of the model of the temporary alternative.

²⁷ It is based on the axioms of usefulness theory.

²⁸ The term appeared for the first time in 1970 and two years later some scholars pointed out that the functional years earned are equivalent to years of additional life assessed for quality. It should be noted that *QALYs* are not all the same, they vary depending on the assessment method (standardized gamble, time trade-off or index systems).

2.4 Research Perspectives of Economic Evaluations

As already mentioned, one of the main problems of public and private healthcare systems is finding and allocating resources. In the coming decades, this phenomenon will be increasingly important for healthcare decision makers given the continued aging of the population, the increase in life expectancy and technological progress.

It can be inferred from what is written, that the basic concept of economic evaluation²⁹ is the “opportunity cost”, that is, the benefit renounced when using the available resources in the best alternative use. After an in depth analysis of the aspects of technical evaluations, from the partial ones to the complete ones, we must reiterate that economic evaluations have limits, the main of which are: poor methodological quality; conflicts of interest (results of efficacy of new drugs and study funding); socio-economic differences in the population assisted, varying appropriateness of the services.

In the design of economic evaluation there are still two delicate points to underline: the choice of the alternative with which to compare and efficacy data under study. The quality of the efficacy data concerns the feedback that should be based on a controlled clinical trial or a systematic assessment.³⁰ In the event that there are no experiments, it is necessary to refer exclusively to the model that includes the limits highlighted before.

The last issue, though not in order of importance, is to decide which costs should be included in economic evaluations. Usually only the direct costs are taken into account if the point of view is the one adopted by the NHS, while the costs called intangible will be taken into account if the point of view is that of patients and company. In the case of diseases with a significant share of indirect costs (migraine, multiple sclerosis...) if the general point of view of the company is not

²⁹ Drummond has examined a number of examples in which economic evaluations are used to sustain some decisions. However, the use of economic evaluations is still limited in relation to its potential. There is a number of underlying reasons for this scant use, among which the lack of dissemination of the results, the lack of credibility and importance of decision makers, the lack of result understanding and the absence of mechanisms for using economic considerations in decision making.

³⁰ Systematic reviews provide a means to efficiently summarize the information on which to base clinical decisions. They have the objective of making the reader aware about the rapid identification of the state of research on an intervention, the evaluation of the methods used in primary studies and the extent of the effect of a treatment in different contexts. In order to decide if there is enough evidence on the effectiveness of an intervention or if it is necessary to conduct further studies to evaluate a treatment and what aspects should be considered. Systematic reviews differ from traditional reviews. These procedures require the author's influence when selecting the area of study, critical evaluation of the studies and the synthesis of the results. Vice versa, systematic reviews follow standard protocols where the basic elements are the completeness of the research studies, assessment of the quality of the studies to be included and the ability to synthesize the results quantitatively through meta-analysis (Dictionary of the New Health Care).

adopted (also considering the indirect costs) it can lead to very different conclusions in than if they are not considered in the analysis.

In order to assess the robustness of economic evaluation results a sensitivity³¹ analysis must be carried out, a process through which all costs and benefits are varied. It will result in a univariate analysis if only one parameter varies and multivariate if more than one parameters vary. In this last case it will be possible to evaluate the best case (lower costs for a more effective new alternative) and the worst case (greater costs and worst effectiveness).

Regarding the results, in a comparative economic evaluation between an innovative treatment A, and an old one B, it may result in the following situations:

- A less effective than B is more expensive and is obviously not taken into consideration
- A more effective than B is less expensive (always will be adopted) DOMINANT
- A less effective than B is less expensive (a little less effective alternative may be chosen if not very expensive)
- A more effective than B but also more expensive. In this case an incremental ratio is carried out that is a cost difference between A and B and an efficacy difference between A and B

Although the futility of certain economic evaluation studies has been demonstrated, Drummond points out that, although there are problems of assessment, there are no real alternatives.

Drummond proposes two things to improve the quality of the work and its transferability into clinical and organizational practice. The first concerns the preferability of economic evaluations based on prospective and naturalistic studies, linked to clinical practice, in which physicians and patients are free to follow their habitual behaviours.

The second proposal made by Drummond regards the main requirement of each cost-effective task, i.e. that the assumptions, models and possible *bias* are well described, transparent and sustained by evidence, the strength of which is open to criticism by any reader.

To this regard, a solution to the problem of quality would be to make all the data available through on line magazines so that readers can analyze and possibly reproduce the results in other contexts. This would make feedback more transparent hence credible.

The analysis of economic evaluation has been developed to provide the public decision-makers with more knowledge in decision allocation. However, even if

³¹ Suppose, for example, that a system of community assistance is set up to prevent unnecessary hospitalization. It is possible if you have home nursing units able to assess the patient's condition and to ensure that they are provided with the service which they actually need. However, it is not clear what would happen to the patient if this type of service were not possible. Therefore, we can try to determine how savings are achieved avoiding unnecessary hospital admissions, altering the parameters related to: cost of patient's hospitalization in the event of admission (cost), number of patients who have been hospitalized in the absence of service (volume).

there has been an increase in the economic evaluation studies both nationally and internationally, little is known about the actual application of such studies in the field of decision-making and the little information collected is not comforting.

The issue is however of great importance, since the future of economic evaluations is closely connected to the use of these studies in decision-making. In Italy, a study, with the objective of analyzing the perception that decision-makers and the general public have of economic evaluations in healthcare, has been carried out. In 2004, the group Cergas Bocconi conducted a study which showed that 65 % of people surveyed (including General Directors of Hospitals, the Departments of Health and Regional Agencies for Healthcare Services) are aware of the evaluation techniques and 85 % believe that the economic evaluations is an essential tool on which to base a decision.

For the application of economic evaluations, Italian decision makers come up against many difficulties due to the complexity of the analysis process and the multiplicity of assumptions to consider.

When it came to gathering advice about greater use of economic evaluations, it was found that the existence of clear guidelines, the inclusion of a financial analysis of budget and the independent researchers' audit work, are among the more reassuring factors.

The creation of guidelines is needed so that economic evaluations appear trustworthy, but even more incisive could be the adequate training of policy makers.

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