Concept of Combining Cost-Effectiveness Analysis and Budget Impact Analysis in Health Care Decision-Making

Roza Ismailovna Yagudina, PhD, Andrey Urievich Kulikov, PhD, Vjacheslav Gennadievich Serpik, PhD, Dzhumber Tengizovich Ugrekhelidze, MSc*

Department of Drug Supply and Pharmacoeconomics, I.M. Sechenov First Moscow State Medical University, Moscow, Russia

ABSTRACT

Objectives: The objective of this study is to cover the ways of solving the problem of understanding the results of two key methods of pharmacoeconomic analysis—budget impact analysis and cost-effectiveness analysis. It is important to note that pharmacoeconomic assessment based on this evidence often has controversial character. The results of one type of analysis can characterize assessed health technology favorably, and the results of other critically. Pharmacoeconomic evidence is often a crucial part of decision-making in healthcare, that’s why clear understanding of combination of this two types of analysis is highly in demand. Methods: Authors propose methodological solution of the stated problem. This model is a useful tool in making unified pharmacoeconomic report based on cost-effectiveness analysis and budget impact analysis results. Use of this model preserves the meaning and significance of each type of pharmacoeconomic analysis. Results: Three-dimensional pharmacoeconomic model proposes full account of both types of pharmacoeconomic analyses during conclusion preparation, the formation of a single consistent pharmacoeconomic conclusion. Though further validation of a tool is needed, presented model can be interesting for the professional community. Conclusions: The proposed model of combining budget impact and cost-effectiveness analysis can be used by healthcare decision-makers for obtaining reliable and transparent pharmacoeconomic data. Keywords: budget impact analysis, cost-effectiveness analysis, health care decision making, health economics, health technology assessment, pharmacoeconomic model.

© 2017 Published by Elsevier Inc. on behalf of International Society for Pharmacoeconomics and Outcomes Research (ISPOR).

Introduction

In this article, the authors propose to consider the possibility of combining the two most widespread methods of pharmacoeconomic analysis—budget impact analysis (BIA) and cost-effectiveness analysis (CEA)—in the same pharmacoeconomic study. Decision making based on pharmacoeconomic evaluations at the state level requires transparent rules and clear definitions of obtained results. The demand for tools of pharmacoeconomic analysis in health care decision making is increasing, mainly because of the high cost of innovative health technologies and limited possibilities of their funding [1]. The BIA and CEA results provide the most important data for health care decision makers. BIAs are an essential part of a comprehensive economic assessment of a health care intervention and are increasingly required by reimbursement authorities as part of a listing or reimbursement submission [2,3]. A BIA is supposed to be complementary to more established types of economic evaluations, mainly CEA, by providing decision makers with additional information on the financial consequences of covering and reimbursing new technologies. Thus, the outcomes of a BIA should reflect scenarios that consist of a set of specific assumptions and data inputs of interest to the decision makers rather than a scientifically chosen “base” or “reference” case as is usually done in a CEA [4]. In particular, both types of pharmacoeconomic analysis are present in the Resolution of the Government of the Russian Federation (No. 871) “On the development of rules of medicinal drugs lists formation” in the section on the requirements to the pharmacoeconomic part of the dossier to the medicinal drug.

Nevertheless, describing the level of pharmacoeconomics implementation in the Russian Federation health care system, it is necessary to highlight the national peculiarities of this process. In several developed countries, such as the United Kingdom, Australia, Canada, and Japan, CEA is the only type of pharmacoeconomic analysis on the basis of which pharmacoeconomic assessment is conducted and which is considered during making decisions about reimbursement of a particular technology by health authorities. Results of the CEA (incremental cost-effectiveness ratio [ICER]) are then compared with the willingness-to-pay (WTP) threshold value. Thus, in developed countries, health care decision makers use only one criterion.
Although there are debates on the correctness of this way of assessment, there is no possibility of any contradiction in the received pharmacoeconomic assessment results. In some countries, including Russia, BIA is widely used in addition to CEA because of the insufficient financing and multisystem health care management system. Furthermore, pharmacoeconomic conclusions obtained using BIA are more valuable for local decision makers as practical experience shows.

The forced necessity of the simultaneous use of two types of pharmacoeconomic analysis creates a potential contradictory situation, in which, for example, evaluation of medicines for the possibility of their inclusion in the state program funding using one type of analysis will be positive, but it will be negative using the other type of analysis. Moreover, according to our experience of conducting pharmacoeconomic studies, there are many times when the same technology is characterized as strictly preferred from the perspective of CEA (i.e., the clinically most effective technology has the minimum value of the cost-effectiveness ratio compared with the alternative), but it is inferior to the alternative according to the results of the BIA (i.e., accompanied by high costs compared with the alternative). Such situations are often encountered in practice [1]. In this connection, the problem of making decisions on the basis of possibly contradictory pharmacoeconomic conclusions arises. In the Russian Federation Government decree “On approval of rules of formation of drugs list,” this problem is solved by the introduction of a scoring system in which the points of a negative opinion of one method can be compensated by the positive points of the conclusions of another method. Nevertheless, the presented approach only formally solves the problem of the conflicting opinions of two different types of pharmacoeconomic analysis, which creates the danger of a misinterpretation (loss of meaning) of the pharmacoeconomic analysis, which creates the danger of a misinterpretation of the conclusions of both types of pharmacoeconomic analysis, BIA and CEA, upon the condition of absence of distortion of these conclusions. It then seems necessary to find an alternative solution to the problem of assessing the findings of both BIA and CEA without misunderstanding these conclusions.

**Methods**

This methodological study was conducted from the perspective of health care decision makers. In the first stage, authors carried out its decomposition, which resulted in the following presentation.

CEA is a pharmacoeconomic method that allows to determine appropriate health technologies using health outcomes criteria (diagnostics, prevention, and rehabilitation) and to determine costs using comparative assessment of outcomes. Costs of two or more health technologies with different effectiveness and results are presented in the same measurement unit [5]. For a visual description of the properties of the CEA conclusions, see Figure 1, which displays a graph and the associated calculations in the form of formulas.

The figure represents a two-dimensional coordinate system in which the effectiveness of the two hypothetically considered health technologies (Ef1 and Ef2) according to selected effectiveness criteria (quality-adjusted life-years and life-years gained) is plotted on the x-axis, and the cost associated with these technologies in monetary terms (Cost1 and Cost2) is plotted on the y-axis. The graph shows that technology 2 with better effectiveness requires higher costs compared with technology 1. Technologies 1 and 2 are indicated by blue points on the graph. In the following step, the points corresponding to technologies 1 and 2 are connected with straight lines from the origin point. Then, the point corresponding to the selected single value of the

**Cost-effectiveness ratio—CER:**

\[
\text{CER} = \frac{\text{Cost}}{\text{Ef}}
\]

**Incremental cost-effectiveness ratio—ICER:**

\[
\text{ICER} = \frac{\text{Cost2} - \text{Cost1}}{\text{Ef2} - \text{Ef1}}
\]

**WTP scale**


Available with limits

Costs max

WTP scale

Costs min

Effectiveness = QALY (LYG)

Costs

Fig. 1 – Graphical representation of cost-effectiveness analysis WTP - willingness-to-pay, ICER - incremental cost-effectiveness ratio, CER - cost-effectiveness ratio, GDP - gross domestic product, QALY - quality-adjusted life year, LYG - life years gained.
effectiveness criteria is plotted on the x-axis, which represents the chosen technology’s effectiveness according to the chosen effectiveness criteria; perpendicularity is restored from this point. Points CER₁ and CER₂ crossing a specified perpendicular with like segments connecting the origin point with the point corresponding to the considered technologies graphically represent the calculated values of the cost-effectiveness ratios of the analyzed technologies. Therefore, the cost-effectiveness ratio describes the cost (in monetary terms) of efficiency unit for each technology. The cost-effectiveness ratio of the more effective technology is lower than that of the less effective one from the perspective of CEA, the more effective technology should be highly preferred. As presented in Figure 1, the more effective technology is characterized by a higher value of cost-effectiveness ratio, which requires conduction of incremental CEA. On the graph, the point corresponding to the ICER is also at the intersection with the restored perpendicular and is shown in purple. The ICER shows the cost of additional efficiency represented by the more effective technology. Its graphical calculation is carried out by the composition of vectors. Then, the segment (highlighted in rainbow color) reflecting the gradation of decisions on the incremental cost-effectiveness values according to the WTP threshold was placed on the perpendicular, restored from a point corresponding to a single value of the effectiveness criteria. WTP is calculated according to Equation 5 (Fig. 1). On the basis of the proposed grading, technology depending on ICER can be characterized as 1) profitable; 2) cost-effective (Fig. 1); 3) available with limits; or 4) unacceptable. Thus, the decision criterion based on CEA has the following significant characteristics:

1. It is a non-negative value.
2. It reflects the value of (additional) unit of effectiveness, ranging health technologies according to the degree of profitability.
3. It is the specific value.
4. It is presented in quantitative monetary terms.
5. Quantitative values are characterized by five quality categories: dominant, profitable, cost-effective, marginally acceptable (possible), and unacceptable.
6. Qualitative categories are more valuable for decision makers.

BIA, in accordance with its name, allows to determine the effects of the implementation of health technologies on the health care budget. BIA begins with determining the total economic effects of the health technology on the health care budget, which represents the algebraic sum of the costs and savings associated with the ones considered by the health technology. The aim of the analysis is to determine the advantages of the health technologies by comparing the calculated values of the total economic effects of each of them.

Technology with lower total economic effects is considered predominant. Nevertheless, it should be noted that representation of the BIA results is more diverse in comparison with that of the CEA results. In addition to the aforementioned “basic scenario” of BIA, this pharmacoeconomic tool allows you to define the possible scope of implementation of the studied technologies on the basis of such conditions of the health system as the amount of available budget and the number of patients in case of full coverage of medical care for all patients. This scenario is called the “optimization scenario” of BIA. BIA also helps to implement the “investment scenario,” which is determined by the required amount of budget to cover the cost of this technology to achieve given target health outcomes in target patient groups [6]. When conducting the innovation or optimization scenario, BIA conclusions are characterized not only by qualitative categories (presence/absence of savings) but also by the accurate quantitative indicators. It is also important to note that these figures are not specific, in contrast to the cost-effectiveness ratio, and therefore these figures tend to vary (and often dramatically) when you change inputs such as the number of patients and the level of technology implementation (which is closely dependent on variable costs).

BIA is graphically presented in Fig. 2, similarly as CEA (Fig. 1). In the figure, BIA corresponds to a one-dimensional structure with scale, with values in monetary terms (Fig. 2).

The green part of the scale reflects the potential savings provided by one of the compared technologies, whereas the additional costs of more expensive technology, the total economic effects of each of the technologies, the budget required to cover the technology to the given population, and the available budget of the health system are reflected on the red part of the scale. Thus, the decision criterion based on the BIA has the following characteristics:

1. It is a non-negative value.
2. It defines various parameters of the budget during the implementation of health technologies.
3. It provides a comparative evaluation of the two technologies from the perspective of their impact on the health budget.
4. It is not the specific value.
5. It has a monetary value.
6. It is more quantitative than qualitative in nature: only for the basic case of BIA, qualitative categorization of the findings is available.

Comparison of the properties of BIA and CEA criteria shows that although the criteria of both are characterized by the quantitative monetary form of expression, the conclusion on the criterion of CEA is more qualitative in nature, whereas the conclusion on the criterion of BIA is quantitative. In addition, sense-bearing differences between the described types of pharmacoeconomic analysis should be noted: if CEA shows the profitability of the particular technology use, the BIA deals with availability of technologies on the basis of the capabilities of the health care system. In this regard, in our opinion, to prevent distortion of meaning of both pharmacoeconomic criteria during decision making, the use of a noncompensatory approach instead of an integral (compensatory) approach in obtaining scores may be more rational. In the proposed approach, the influence of each criterion (CEA and BIA) will be considered independently. This allows to preserve the logic of methods. The proposed noncompensatory approach is based on the idea of importance for decision makers. It helps to highlight areas characterizing the possibility of the health technologies approval or disapproval from the pharmacoeconomic analysis perspective.
In fact, selection of pharmacoeconomic index boundaries characterizing the considered health technology out of scope of which technology will be definitely denied may be most relevant in the first stage. After determining the boundaries of this area, ranking of analyzed technologies according to optimality of pharmacoeconomic profile should be done in case the analyzed ones match in the approval area. Then, taking into account the high level of relevance of BIA and CEA in Russian conditions, the boundaries of the specified area should be formed according to the respective values of the two listed pharmacoeconomic tool indicators.

Considering that CEA can be presented in a two-dimensional spatial representation and BIA in a one-dimensional one, construction of a united area of health technology approval on the basis of two listed criteria involves using a three-dimensional structure (Fig. 3).

In Figure 4, the health technology is described in the three-dimensional coordinate system from the perspective of two pharmacoeconomic criteria, the axes of which correspond to the effectiveness, costs, and results of the BIA. The approval of health technologies in accordance with the described approach is defined by three half-planes: I, II, and III (Fig. 3). The first half-plane reflects a health system capacity limit of payment for technologies; the second shows the limitations imposed by the WTP threshold in relation to the ICER, and the third obviously follows from the impossibility of negative effectiveness axis values (because the point of origin corresponds not to the current practice and efficiency report in relation to it, but to 0 values of all three axes).

The proposed approach can be illustrated in a hypothetical case of the inclusion of the medicinal drug $A$, which is used for treatment of disease $M$, in the reimbursement list. Drug $A$ is characterized by the value of the ICER $\€13,567$ per quality-adjusted life-year, and the total cost of treating patients with drug $A$ for a given level of market share is $\€178.38$ million. Health care budget includes $\€125$ million for reimbursement costs of this drug. The WTP threshold in the example is $\€20,000$. This information can be used as follows in the described three-dimensional model. From the point of view of the mathematical expression, the specified methodological approach, in fact, can be described by a system of inequalities:

\[
\begin{align*}
\text{ICER} & < \text{WTP}, \\
\text{BIA} & < \text{Constant},
\end{align*}
\]

where ICER is the incremental cost-effectiveness ratio, WTP is the willingness-to-pay value, BIA represents the result of BIA, and Constant represents the maximum amount of available budget.

If both conditions of the system of inequalities are fulfilled, then drug $A$ is in the area of inclusion in the reimbursement list, whereas in the opposite case, it is in the area of decline.

On entering values for the described hypothetical situations in the inequality of Equation 6, we obtain:

\[
\begin{align*}
\€13,567 < \€20,000 & \Rightarrow \text{fulfilled} \\
\€178.38 \text{ million} < \€125 \text{ million} & \Rightarrow \text{fulfilled}.
\end{align*}
\]

As it follows from the presented data, the conditions of the system of inequalities are not fulfilled; therefore, the authors concluded that drug $A$ was in the area of rejection of its inclusion in the reimbursement list.

As an example from the actual practice of the described approach, we analyzed the possibility of inclusion of the medicinal drug ibrutinib in the state program of preferential medicinal maintenance of patients with hemophilia, cystic fibrosis, pituitary dwarfs, Gaucher disease, malignant neoplasms of lymphoid, hematopoietic and related tissue, and multiple sclerosis, and persons after transplantation of bodies and/or tissues (seven high-costly nosologies) in the Russian Federation [7] in the treatment of chronic lymphatic leukemia (CLL). The selection of this program for our example can be explained by the presence of an approved fixed budget for its financing. For this, we used the results of two previously conducted pharmacoeconomic studies of ibrutinib including cost-effectiveness [8] and BIA data [9]. In the CEA study, the following treatment schemes of CLL were compared: ibrutinib and bendamustine + rituximab. The values of the cost-effectiveness ratio of using the criterion of effectiveness of survival without progression were $€72,542$ for ibrutinib and $€76,383$ for bendamustine + rituximab for 1 year without progression of the disease [8] ($€1 = 69.63$ nuble). Clinical efficacy of ibrutinib and bendamustine + rituximab in the treatment of CLL, expressed as survival without progression, was 30 months and 15.2 months, respectively [10,11]. The cost of treating one patient with CLL on the drug ibrutinib was $€181,356$, and on bendamustine + rituximab it was $€96,752$ [8]. The BIA results of ibrutinib in the treatment of patients with CLL showed that when the market share of ibrutinib equals 23% (1335 patients), the required budget will be $€46.96$ million [9].

In this example, the ICER of the drug ibrutinib is characterized by a lower value of the cost-effectiveness ratio and higher clinical efficacy and equals $€5,716$, whereas the gross domestic product in Russia in 2016 amounted to $€27,707$ [12].

During checking the second inequality presented in Equation 6 of the system of inequalities, the difficulty arises because of the need to set the value of Constant.

In practice, the relevant executive authorities determine a limit of the available budget. In this example, authors determined the value of Constant on the basis of the condition of no increase in the existing budget of the seven high-costly nosologies program. Given this condition, the value of Constant was determined with internal reserves of the program generated through a factor of the expiration of patent protection for original drugs.

---

**Fig. 3** – «3D» pharmacoeconomic model of health technology approval area used in their inclusion in state funding programs based on pharmacoeconomic criteria: cost-effectiveness analysis and budget impact analysis. BIA - budget impact analysis.
included in the program, and accordingly the output of generic drugs and the projected decline in prices. The value of Constant for the calculations amounted to €49.83 million [13]. Thus, the system of inequalities represented in Equation 6 on the basis of our example is the following:

\[
\begin{align*}
& 5,716 < 27,707; \\
& 46.96\text{ million} < 49.83\text{ million}
\end{align*}
\]

Consequently, both the conditions of inequalities are fulfilled and the drug ibrutinib for the treatment of CLL is in the area of approval when making decisions.

**Results**

The article presents the concept of a practical embodiment of the described methodology—the creation of a three-dimensional pharmacoeconomic model. It is a useful tool for making consistent pharmacoeconomic reports on the basis of CEA and BIA results, which allows to reflect the significance of each type of analysis. For the first time in Russia it was developed and presented as the method of health care decision making using the criteria of the two types of pharmacoeconomic analysis (CEA and BIA). Characterized by the number of advantages (full account of results of both types of pharmacoeconomic analyses during conclusion preparation; formation of a single consistent pharmacoeconomic conclusion; and further ranking possibility of the discussed technologies), the technique requires further validation and verification of applicability in real conditions; nevertheless, the authors hope that this research will be interesting to the professional community and will initiate a discussion on this topic.

**Discussion**

In the modern conditions of health development, the principal significance is addressed to the effective use of the available resources, which is especially important when choosing health technologies (medicaments, medical devices, information technologies, etc.) provided by the state budget. Optimal management decisions in this area should ensure the achievement of these goals using minimally adequate resources in relation to the specific local conditions of the health system, whether on federal and regional levels or applied to individual hospitals. According to experience, the procedures of regular managerial decision making require auxiliary (supporting) tools to provide the decision makers with the necessary information. Currently, the health care system of the Russian Federation has gone through the stages of systematic changes to meet the modern requirements and realize the settled objectives. Nevertheless, the mentioned changes are not only limited to the incorporation of innovative prophylactic, medical-diagnostic, and rehabilitative technologies, but they also affect the areas of management and decision making in the health system. One of the most important tips in the health system management is the development of rules/algorithms including medical technologies in public health programs.

**Conclusions**

The proposed method of combining budget impact and cost-effectiveness analysis is a unique tool for elevation of accuracy and quality of pharmacoeconomic analysis and health technology assessment. Further discussion can improve the way of implementation of this method.

Source of financial support: No funding was received for this study.

**References**


