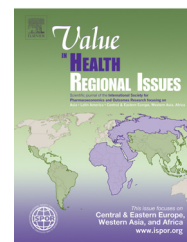




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## Cost-effectiveness versus Cost-Utility Analyses: What Are the Motives Behind Using Each and How Do Their Results Differ?—A Polish Example

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### ABSTRACT

**Objectives:** We aimed to compare the use of cost-effectiveness analysis and cost-utility analysis in health technology assessment in Poland. **Methods:** We analyzed all the submissions (155) made to the Polish Agency for Health Technology Assessment in the period 2007 to 2011, with 316 intervention-comparator comparisons reporting incremental cost-effectiveness ratios (ICERs) or incremental cost-utility ratios (ICURs). We compared ICERs and ICURs when both were reported (31%), determined factors associated with reporting one or the other, and tested the precision of their assessment. **Results:** In 13% of the cases, ICER and ICUR led to different decisions (were on opposite sides of the willingness-to-pay threshold). Cost-effectiveness analyses were more frequently performed in oncology, offering at the same time more favorable results. It was also more frequent for longer time-horizon models, although then ICER values were on average higher. **Conclusions:**

In Poland, cost-utility analysis is a usual approach of increasing popularity. Interestingly, although assessing ICUR requires additional assumptions, it is estimated more precisely (reported ranges of values in sensitivity analyses are narrower), especially in oncology. ICER and ICUR disagree more often than previously shown in literature. There seem to be no clear signs of biases in submissions (selecting whether to present ICER or ICUR on the basis of their values), but the current study is limited because only the values presented by manufacturers in the submission are available.

**Keywords:** cost-effectiveness analysis, cost-utility analysis, HTA, incremental cost-effectiveness ratio, incremental cost-utility ratio, Poland.

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### Introduction

Health technology assessment (HTA) reports presenting the rationale to reimburse a new health technology typically encompass an economic analysis, that is, quantification of additional cost of using this technology in relation to additional health effects [1,2]. Health effects are usually measured as life-years gained (LYGs) or quality-adjusted life-years (QALYs), accounting also for the quality-of-life outcomes [3]. An economic analysis in which LYGs are used is often referred to as cost-effectiveness analysis (CEA) with its parameter of interest being called incremental cost-effectiveness ratio (ICER), whereas an analysis in which QALYs are used is often called cost-utility analysis (CUA) and the resulting parameter is called incremental cost-utility ratio (ICUR). The terms ICER and ICUR are sometimes not distinguished and the context tells whether the result is expressed in LYGs or QALYs. The ICER/ICUR is then compared with the (official or approximate) willingness to pay (WTP) for a unit of effect, that is, threshold to make a final recommendation.

Because HTA aims to evaluate the complete economic and clinical consequences, it would seem natural to favor CUA over

CEA. Indeed, although national HTA guidelines differ and may be sometimes vague (cf. Table 1), CUA is overall preferred (strongly preferred in six countries, somewhat preferred in two, not preferred in five). Agency for Health Technology Assessment in Poland (AHTAPol) guidelines treat CEA and CUA equally, demanding that the choice between them be justified; however, the Polish Reimbursement Act, which came into force in 2012, strictly prefers CUA.

This article aims to compare the use of CEA and CUA in HTA in Poland via econometric analysis of data in submissions. In particular, we intend to analyze 1) what leads to the selection of CEA or CUA; 2) how their results differ (point values and range in sensitivity analysis); and 3) whether any bias is present, that is, preferring a more favorable type of analysis to present in a submission.

Our analysis can help to answer the question whether such regulations, obliging manufacturers to present CUAs, were needed in Poland (e.g., when there seems to have been some bias in selecting CEA or CUA) and how they can affect the natural path of HTA development. Such analysis may support other countries in the Central and Eastern European region in shaping their formal HTA regulations.

Conflicts of interest: The authors have indicated that they have no conflicts of interest with regard to the content of this article.

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**Table 1 – Summary of selected national guidelines for the form of economic evaluation.**

Country, institution; guidelines, year	Recommendations for the analytic method in economic evaluation	CUA preferred over CEA?
Australia, Pharmaceutical Benefits Advisory Committee; Guidelines for Preparing Submissions to the Pharmaceutical Benefits Advisory Committee, 2008 [4]	<p>“Cost-utility analysis (generally preferred). (...), a cost-utility analysis is the preferred form of economic evaluation for either or both of the following situations:</p> <ul style="list-style-type: none"> <li>- where there is a claim of incremental life-years gained in the economic evaluation - in order to assess the impact of quality adjusting that survival gain</li> <li>- where relevant direct randomised trials report results using a MAUI.”</li> </ul>	Yes, CUA generally preferred.
Belgium, Belgian Health Care Knowledge Centre; Belgian Guidelines for Economic Evaluations and Budget Impact Analyses: Second Edition, 2012 [5]	<p>“Cost-effectiveness analysis should be used if improving life expectancy is the main objective of the treatment (...). Cost-utility analysis should be used if the treatment has an impact on health-related quality of life that is significant to the patient or if there are multiple patient-relevant clinical outcome parameters expressed in different units. If a cost-utility ratio is presented as a reference case analysis result, the corresponding cost per life-year gained should also be presented.”</p>	No. CUA preferred if the treatment has an impact on HRQOL. Should be accompanied by CEA.
Canada, Canadian Agency for Drugs and Technologies in Health; Guidelines for the Economic Evaluation of Health Technologies, 2006 [6]	<p>“A CUA should be used in the Reference Case where meaningful HRQL differences between the intervention and alternatives have been demonstrated, and where appropriate preference (utility) data are available. A CEA should be used as the Reference Case when a CUA is an inappropriate choice. Use a final outcome (e.g., life-years gained), or if that is impossible, an important patient outcome. (...)”</p>	Qualified yes.
France, Collège des Économistes de la Santé; French Guidelines for the Economic Evaluation of Health Care Technologies, 2004 [7]	<p>“Each evaluation has its own particular scope and limitations. The type of study selected should be clearly stated and justified with respect to the issue addressed, and must be described at the start of the study. The author should also provide his personal definition of the type of study used.”</p>	No, it depends on the case.
Finland, Ministry of Social Affairs and Health; Guidelines for Preparing a Health Economic Evaluation, 2009 [8]	<p>“The choice of the method of analysis most suitable for each situation (cost-minimization analysis, cost-effectiveness analysis, cost-utility analysis and cost-benefit analysis) depends primarily on how the therapies compared affect patients’ health state.”</p>	No, it depends on the case.
Ireland, The Health Information and Quality Authority; Guidelines for the Economic Evaluation of Health Technologies in Ireland, 2010 [9]	<p>“The preferred evaluation type for the reference case is a cost-utility analysis (CUA) with the outcomes expressed in terms of quality-adjusted life-years (QALYs). In exceptional circumstances, a cost-effectiveness analysis (CEA) with the outcomes expressed in terms of life-years gained (or other relevant outcome if the technology does not add life-years) may be used as the reference case when a cost-utility analysis is an unsuitable choice. Clear, detailed empirical evidence must be provided to justify this position.”</p>	Yes.
The Netherlands, College voor zorgverzekeringen; Guidelines for Pharmacoeconomic Research, 2006 [10]	<p>“If the improvement in quality of life forms an important effect of the drug being assessed, then it is necessary to carry out a cost-utility analysis (CUA). If this is not the case, then a cost-effectiveness (CEA) has to be carried out. (...)”</p>	No, it depends on the case.
New Zealand, Pharmaceutical Management Agency (PHARMAC); Guidelines for Funding Applications to PHARMAC, 2010 [11]	<p>“Economic analyses should be in the form of a CUA, with benefits measured in terms of quality-adjusted life-years (QALYs). In cases where the clinical outcomes of the drug and the comparator have been shown to be equivalent, a cost-minimisation analysis may be appropriate. Other forms of cost-effectiveness or cost-benefit analyses (CBA) should not be provided to PHARMAC.”</p>	Yes.

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