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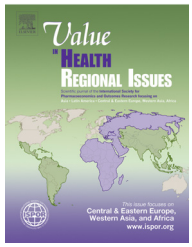

Health Economic Data Requirements and Availability in the European Union: Results of a Survey Among 10 European Countries

Jana Skoupá, MD[1,*], *Lieven Annemans, PhD*[2], *Petr Hájek, MSc*[3]

[1]*Medical Data Center, 1st Medical Faculty, Charles University, Prague, Czech Republic;* [2]*Department of Public Health, University of Ghent, Ghent, Belgium;* [3]*Pfizer s.r.o, Prague, Czech Republic*

ABSTRACT

Objectives: To compare data requirements and their availability for health economic (HE) evaluations in five countries in Central/Eastern Europe (CEE) (Poland, the Czech Republic, Slovakia, Hungary, and Romania) and five countries in Western Europe (WE) (the United Kingdom, France, Germany, The Netherlands, and Sweden). **Methods:** A questionnaire was developed and distributed to market access personnel from Pfizer who were asked to complete the questionnaire either from their own knowledge or with support of external experts. The questionnaire focused on the obligation to conduct HE assessment for reimbursement submissions, local HE guidelines, applied discount rates for future costs and effects, willingness-to-pay thresholds, and available data sources. **Results:** HE is mandatory in all CEE and three WE participating countries for reimbursement applications of innovative drugs. Usually, cost-effectiveness analysis and budget-impact analyses are required. The preferred outcome of cost-effectiveness analysis is quality-adjusted-life years. In Romania, France, and the Czech Republic, guidelines could not be identified at the time of the survey. The applicant usually prepares HE evaluations; in Sweden, the United Kingdom, The Netherlands, and Poland, unlocked models have to be presented for scrutiny. Discount rates vary from 1.5% to 5%, and, usually, is the same for costs and outcomes (except in The Netherlands and Poland). Only the United Kingdom, Poland, and Slovakia have an explicit willingness-to-pay threshold. In Poland, it is based on the gross domestic product per capita, and in Slovakia, it is based on multiples of average monthly salary. Differences were found on data availability. In WE, data can be acquired easier than in CEE. Health insurance funds do not provide their data unless they were published. Patient registries are either not available in CEE or difficult to access, so applicants mostly rely on retrospective medical chart data, hospital information systems, or expert panels. **Conclusions:** We found similar requirements for HE analyses in CEE and WE but differences in data availability. This results in less accurate inputs across the CEE, influencing analyses' outcomes.

Keywords: Central and Eastern Europe, cost-effectiveness, data availability, data requirement, health technology assessment, Western Europe.

Introduction

The continuous influx of health care technologies across European Union (EU) member states together with limited financial resources have put more emphasis on identifying those innovations providing best value for money. Structured health technology assessment (HTA) has therefore been implemented across Europe. Cost-effectiveness (CE) and also budget impact (BI) analyses represent an important part of HTA in most of the countries, including Central and Eastern European (CEE) countries. Hence, not surprisingly, CE and BI analyses are rapidly emerging in these countries.

But CE studies and BI analyses require data, and the general feeling is that such data are much less available in CEE countries than in Western European (WE) countries. We conducted a survey among 10 CEE and WE member states, with the aim of comparing data requirements and their availability for health economic (HE) evaluations.

Methods

A total of 10 countries participated in the survey, five representing Central and Eastern Europe (the Czech Republic, Hungary, Poland, Romania, and Slovakia) and five representing Western Europe (France, Germany, The Netherlands, Sweden, and the United Kingdom). The project was supported by an educational grant from Pfizer.

A common 11-item questionnaire was developed and sent out to participating countries (see Appendix 1 found in Supplemental Materials found at http://dx.doi.org/10.1016/j.vhri.2014.06.003). Health economics and outcomes research representatives from Pfizer in individual countries were asked to complete the questionnaire either from their own knowledge or with the support of local experts. Data obtained were synthesized and rechecked locally by local experts in HTA to avoid bias or misinterpretations.

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

* *Address correspondence to:* Jana Skoupá, Pod Šmukýřkou 2, 150 00 Praha 5, Czech Republic.

E-mail: jana.skoupa@lf1.cuni.cz

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The questionnaire included the following items:

1. CE and BI analyses—mandatory/voluntary part of the reimbursement submission.
2. Presence/absence of an official HTA agency.
3. CE and/or BI analyses included/not included in legislation.
4. Methodological guidelines for CE analyses present/absent.
5. Presentation of unlocked CE models to decision makers obligatory/optional.
6. Required perspective of CE analyses—health care/societal.
7. Discount rate for costs and benefits in CE analyses.
8. Choice of the comparator used in the CE analyses.
9. CE threshold.
10. Availability of data sources used in CE and BI analyses including epidemiology, resource utilization, and costs.
11. Unavailable or difficult to obtain data necessary for CE and BI analyses; ways how to overcome missing data.

Results

CE analysis is a mandatory part of the HTA for innovative drugs in all CEE and three of the participating WE countries (The Netherlands, the United Kingdom, and Sweden). BI analysis is usually also required, with the exception of Romania and Sweden, where only CE analysis is mandatory.

All survey participants from WE countries have an official HTA agency, a review body that reviews and/or produces and disseminates assessment reports on medical technologies. Most of the agencies are governmental institutions. In the CEE region, only Poland and Hungary reported an official and functioning governmental agency (note that during 2013, Romania established a formal HTA agency at the Ministry of Health). In addition Poland has several private entities that focus on providing not only HTA reports, systematic reviews, data collection, and evaluation but also training and educational activities.

With the exception of Germany, HE is included in the legislation of all participating CEE and WE countries, usually as a part of a complex HTA process. In France, the reimbursement decision is currently mainly based on the medical benefit of the assessed intervention; for the pricing decision, BI is taken into account. Furthermore, CE data are required only during reassessment after a product is launched. Changes are, however, discussed; that is, CE should be included into the assessment and appraisal process upfront. In most of the other countries, CE is an important parameter for the reimbursement decision (the United Kingdom, Poland, The Netherlands, Sweden, the Czech Republic, and Slovakia). Besides the clinical evaluation (safety, efficacy, and effectiveness) of the assessed technology, its BI and CE are usually considered before reimbursement is granted. Quality-adjusted-life years (QALYs) seem to be the preferred outcome in CE analyses not only in WE countries (the United Kingdom, The Netherlands, and Sweden) but also in several CEE countries (Poland, Slovakia, Hungary, and the Czech Republic).

Not all countries applying HE evaluations in the decision-making process follow local guidelines for HE analyses. In Romania and the Czech Republic, official guidelines could not be identified at the time of the survey (note that in 2013, CE and BI guidelines were published in the Czech Republic by the local pricing and reimbursement authority).

Reimbursement files that include HE evaluations are usually prepared by the applicant (industry) and submitted to local agencies. In Sweden, the United Kingdom, The Netherlands, and Poland, full, unlocked models have to be presented for scrutiny. In CEE countries except Poland, the presentation of models is not obligatory, but when they are used they have to be described in detail and applicants might be asked for clarification or additional information requested by authorities. Differences also exist in the communication between applicants and authorities during the HTA process across countries. While in several countries, applicants are given the opportunity to present HE results to a multidisciplinary committee (Slovakia) or clarify uncertainties in written form (the United Kingdom and Poland), other agencies do not organize hearings (the Czech Republic, The Netherlands, Romania, Poland, and Hungary). In Sweden, applicants might be invited in specific situations, while in The Netherlands scientific advice might be obtained before submitting an official reimbursement dossier.

Only 4 of the 10 countries require a comprehensive societal perspective in the CE analysis (The Netherlands, Sweden, France, and Poland); however, in other countries, societal costs are optional (Romania and the Czech Republic), without an impact on the decision.

The most common annual discount rate used in the CE baseline scenario varies between 1.5% and 5% (Table 1) and, with the exception of The Netherlands and Poland, is the same for costs and outcomes.

One of the crucial parts of the CE analysis is the comparator's choice because all countries use a comparative assessment. In most countries, the comparator is the standard intervention, which is expected to be replaced by the new technology (The Netherlands, the United Kingdom, Sweden, Poland, Hungary, and Slovakia) or the most cost-effective technology, if more comparators can be considered. In some countries, the comparator is either not specified (the Czech Republic, France, and Romania) or explicitly identified by authorities (Germany). Although preliminary advice on comparator selection is possible in some countries (e.g., The Netherlands), the final decision on the appropriate comparator's choice is made during the HTA process (Germany, The Netherlands, Sweden, and the Czech Republic).

Only three of the assessed countries have an explicit willingness-to-pay (WTP) threshold. In the United Kingdom, the threshold of £20000 to £30000 is currently applied with some exceptions (e.g., end-of-life interventions). In Slovakia, a WTP threshold based on multiples of average monthly wage was introduced and is included in legislation. If interventions prove an incremental cost-effectiveness ratio of less than 24 average wages (~€18,500/QALY), they are considered as cost-effective and quite likely to be included into reimbursement lists. The range of 24 to 35 average monthly wages (about €27,000/QALY) enables conditional reimbursement for 2 years with an agreed budget cap. Technologies with a higher incremental cost-effectiveness

Table 1 – Commonly used discount rates for costs and benefits.

Countries	Discount rates for future costs and health benefits (%)	
	Health effects	Future costs
The Netherlands	1.5	4
The United Kingdom	3.5	3.5
Sweden	3	3
France	NA	NA
Germany	Not defined	Not defined
Romania	Not defined	Not defined
Poland	3.5	5
Hungary	3.7	3.7
The Czech Republic	3	3
Slovakia	5	5

NA, not available/applicable.

ratio are reimbursed only if disease prevalence is low (1:100 000). In Poland, the CE threshold is defined as three times the gross domestic product (GDP) per capita; for referencing purposes, a mean of 3 years is used. Several other countries apply an implicit threshold, which is usually between two to three times the GDP per capita (Hungary and The Netherlands). In some countries, the applied threshold value differs on the basis of the type of intervention or a threshold is not defined at all (France, Germany, Romania, and the Czech Republic).

Significant differences across analyzed countries were found in data availability. The survey found that in WE countries, data can be acquired more easily (although not free of charge) than in CEE countries because WE countries' databases on drug prices/reimbursement are available and recent. Data on overall mortality are usually available with a 1- to 2-year delay. Selected data on specific mortality and morbidity can be estimated from governmental sources; however, databases do not include all medical conditions. Health insurance funds usually do not open their data to the public regardless of the considered region. Insurer's data can usually be obtained only from the published literature (Poland and the Czech Republic) or purchased (Hungary). Patient registries are an important source for assessing effectiveness. In WE countries, registries are more accessible (the United Kingdom, The Netherlands, France, Sweden, and Germany) than in CEE countries, where registries are either not available (Romania and Hungary) or not easily accessible (Poland, the Czech Republic, and Slovakia). In the two latter countries, some initiatives are currently underway to enable better utilization and broader access for specified HTA bodies and manufacturers.

Hospital records on epidemiology and patient-reported outcomes are available with restrictions in the United Kingdom, France, and The Netherlands (hospital products only). In the CEE countries, hospital data availability depends on therapeutic area (Hungary), can be obtained by retrospective studies from medical charts and/or hospital information systems (Poland and the Czech Republic), or by expert interviews (Hungary).

For resource use utilization and costs, there are some sources of information available. In all WE countries, recent data are available and free of charge for inpatient data, outpatient fees and reimbursement for procedures, testing, and other diagnostic methods. In CEE countries, data for diagnostic tests and outpatient visits can be derived from published price-lists (annually in the Czech Republic, occasionally in Poland), and in specific circumstances from insurance funds (Hungary and Slovakia) or not available at all (Romania). Obtaining information from insurance funds—charged amounts for health care services—is stated as "difficult" or "unavailable" across all assessed countries.

In WE and CEE countries, the following items are perceived as missing for conducting high-profile HE analyses:

- Specific cost and resource-use data linked to diagnosis (Romania, Slovakia, and partly Sweden)
- Local quality-of-life data (The Netherlands, the United Kingdom, and the Czech Republic)
- Local data on the epidemiology of disease (the United Kingdom, Romania, and Slovakia)
- Accessibility of registry data (Hungary)
- Local HE data (France, Poland, and Slovakia)
- Lack of data on rare diseases (most of the participants including the United Kingdom and The Netherlands)

Because pharmaceutical companies have to overcome the hurdles that exist, they often have to initiate their own research (Table 2).

Discussion

Limited financial resources for health care are a hot topic across the EU. A number of initiatives mainly affecting the pharmaceutical sector have been undertaken to increase the transparency of pricing and reimbursement processes [1], while others focus on best practices in HTA [2]. The "Joint EC (ECFIN)-EPC Report on Health Systems" and the Economic Policy Committee have stressed the need to keep health care budgets under control by rational use of pharmaceuticals [3].

Almost all EU member states regulate pharmaceutical markets to a certain extent. These regulations mainly focus on pricing, reimbursement, market entry, and/or expenditure control. HTA is used to guide pricing and reimbursement decisions. HTA is now used in most of the EU member states as a valuable tool for evidence-based decisions to identify health care interventions perceived to offer greatest value for money. Even in countries not yet following a systematic HTA approach (e.g., the Czech Republic and Romania), steps are undertaken to introduce a regular HTA assessment and appraisal process. Although clinical data might be transferable across Europe, economic data have to reflect specific situations of individual member states and therefore usually cannot be nor should be transferred even from countries with similar socioeconomic attributes. Therefore, local data are needed to obtain robust economic analyses.

Examples from several countries show that building capacity for HTA implementation is an important but costly and long-term process. In Hungary, such a process started in the mid-1990s

Table 2 – Steps usually taken to overcome missing data for health economic analyses.

Countries	Ways to overcome limited data sources				
	Own epidemiology study	Physician surveys	Patient chart reviews	Purchasing data from third party	Organize own registries
The Netherlands	✓	✓	✓	✓	✓
The United Kingdom	✓	✓	✓	✓	✓
Sweden		✓	✓	✓	
France	✓	✓	✓	✓	✓
Germany	✓	✓	✓	✓	✓
Romania	✓	✓		✓	✓
Poland		✓	✓		
Hungary		✓	✓	✓	
The Czech Republic	✓	✓	✓	✓	✓
Slovakia	✓	✓	✓	✓	✓
Total	7	10	9	9	7

and by 2010 the number of trained and experienced professionals exceeded 200 [4].

Because of the recent HTA developments across the EU, we conducted a survey of official requirements and available local data to fulfill these requirements, among 10 EU countries, covering both WE and CEE countries. We specifically also focused on the role of HE assessments (CE and BI) in the countries included in the survey. We found that in our sample economic requirements are much stronger in CEE countries (mandatory in all) than in WE countries (only three countries with mandatory economic evaluation for innovative drugs). In the survey, we did not focus on qualitative aspects of economic applications and assessments; however, the published literature stresses the limited number of trained HTA professionals in CEE countries, lack of local postgraduate university training opportunities, and calls on more action to ensure sufficient HTA capacity [5]. The high unmet need in this respect becomes even more urgent because all CEE countries and most of the WE countries (except Germany) have included HE in their legislation. A closer cooperation with WE universities might be the first step for CEE countries to obtain expertise not only in economic evaluations but also in clinical aspects of HTA. A recent publication compared 48 Polish and Scottish HTA recommendations on the same drugs assessed in the period January through December 2008 [6]. A higher proportion of drug technologies obtained a negative recommendation in Poland (19 of 48) than in Scotland (11 of 48). Surprisingly, clinical reasons for rejection (poor safety and efficacy) dominated decisions in Poland, while economic aspects were most often stated in Scotland. The author herself did not have a clear explanation for this phenomenon; however, she concludes that improvement in Polish methodological guidelines, related to clinical effectiveness issues, is needed to capture patient subpopulations that might benefit from interventions if analyzed separately.

The WTP threshold and outcome measures are other aspects with large variations across the EU. Most of the CEE countries refer to QALY as the appropriate and preferred outcome, showing a strong orientation to the National Institute for Health and Care Excellence and the Scottish Medicines Consortium. Latest developments in the United Kingdom with the planned introduction of "value-based-pricing" as well as the "QALY controversy" discussed recently might question the strict QALY orientation [7]. In countries with an existing threshold value or range, usually the approach connected to GDP is used. It is, however, usually lower than the three times GDP per capita recommended by the World Health Organization [8,9] (for poor countries). Two countries in the CEE (Poland and Slovakia) even have a threshold range strictly incorporated in their legislation. In several WE and CEE countries (France, Germany, Romania, and the Czech Republic), no official threshold was defined although CE analysis is sometimes mandatory for innovative drugs during the reimbursement process (the Czech Republic and Romania). Experience from different jurisdictions reveals the difficulty in justifying a single explicit threshold without denying the option to incorporate other dimensions (as societal preferences) into the pricing and reimbursement process [10,11]. Even if a WTP range is considered, it remains questionable whether equity of access to medicines across citizens can be ensured in an EU environment with differing national economic output and unified pricing strategies set by manufacturers. An analysis, derived from phase III trials, focusing on modern oncology drugs was recently performed in the Czech Republic [12]. Only 4 of the 14 assessed drugs (mainly biological therapies) met the implicit threshold for cost per life-year-gained.

One of the significant differences found in our survey related to data availability. Findings show that access to data seems to be easier in WE countries than in CEE countries, although areas for improvement are found across the whole EU. Epidemiological data, if available, usually have delayed publication, and even health insurance funds do not present sufficient information to be used for economic analyses. Registries seem to be more easily accessible in WE countries, whereas in the CEE countries they are either not available or not easy to access. Exceptions exist such as the Czech National Oncology Registry, which provides a lot of useful information especially on epidemiology and helps to predict expenditure dynamics for oncology treatments. In contrast to WE countries, little effort has been undertaken by governments in the CEE countries to establish registries and use them to show the real-life effectiveness of medications beyond a clinical trial setting for multitechnology appraisals. One of the reasons might be the absence of financial support to create and conduct registries, which are mainly financed by the pharmaceutical industry, together with the complexity of the process [13,14].

Our survey tried to capture differences and similarities across selected EU countries from WE and CEE countries regarding HTA, focusing on HE, which is evolving rapidly in CEE countries. However, our study has several limitations. One of them is the narrow time frame of the survey (last quarter of 2012). In the Results section, we mentioned that CE guidelines are not in place in the Czech Republic and Romania. Meanwhile, a guideline was published and has been effective since February 2013 in the Czech Republic, and Romania is currently exploring possibilities to introduce certain HTA criteria. A further explanation as to why Pan-European decisions about medical technologies are not realistic lies in differences in the financing and organization of health care systems and specific local factors.

Despite the differences, there are several efforts that could be undertaken at the European level and adopted locally. We suggest that clinical HTAs in particular could to a large extent be transferable among countries. A current initiative by EUnetHTA - Joint Action 2, which covers the period of 2012 to 2015, facilitates international collaboration and strengthens the practical application of HTA tools and approaches. This represents a valuable opportunity for CEE countries to get involved in HTA processes and learn from HTA-mature countries.

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Supplemental Materials

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