ISPOR TASK FORCE ON USE OF PHARMACOECONOMIC/
HEALTH ECONOMIC INFORMATION
IN HEALTH CARE DECISION-MAKING

Draft for Discussion*

November 20, 2001

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* This report has been produced by the Core Group Members of the Task Force. It should be viewed as a preliminary draft. We now welcome comments from the Task Force Reference Group and the broader ISPOR membership. A revised version will be produced based on the comments received.
1. **INTRODUCTION**

Despite the growing number of pharmacoeconomic/health economic (PE/HE) studies and the methodological developments in recent years, very little is known about the attitudes of decision-makers to such studies. That is, are studies used and, if so, do they have any impact on decision-making?

Although PE/HE studies can be undertaken for academic interest, their main purpose is to help those making decisions about the allocation of healthcare resources. Therefore, the objectives of this Task Force are to ensure that the good research practices of PE/HE studies pay attention to the needs of healthcare decision-makers and to develop a ‘toolbox’ for the healthcare decision-maker wishing to interpret and use PE/HE studies.

The report is organised in the following manner. First, the contexts for healthcare decision-making and the uses of PE/HE studies are discussed. Secondly, the current literature on decision-makers’ attitudes to PE/HE studies is reviewed. Thirdly, elements of good practice in the reporting of PE/HE studies are specified. Finally, several issues for further research are outlined.

2. **CONTEXTS FOR DECISION-MAKING AND THE USES OF PE/HE STUDIES**

There are several contexts for healthcare decision-making, which may vary from place to place. First, at the *central level*, decisions are made about policies and
programmes for the populations of particular countries or regions. In some jurisdictions these include centralized procedures for the pricing and reimbursement of pharmaceuticals (e.g., Australia, Ontario). In a wider range of jurisdictions there are national programmes for prevention of disease, including screening and immunization.

Secondly, many policy decisions are made at the local level, namely the health plan, hospital or practice. These may include the adoption of treatment guidelines or the inclusion of drugs on the local formulary. In some countries, such as the USA, the majority of healthcare resource allocation decisions are made at the local level.

Finally, healthcare resource allocation decisions are made in all healthcare systems at the patient level. However, in general the main application and relevance of PE/HE studies is at the central and local levels, although these decisions undoubtedly condition the treatment decisions taken by doctors on behalf of their patients. For example, if a given drug is not on the local formulary, or is at the third tier attracting a high patient co-pay, physicians are less likely to recommend it for their patients.

Whereas the same elements of good practice apply to PE/HE studies irrespective of the level of decision-making they seek to inform, there are critical differences between the central and local levels that bear on the work of this Task Force. First, at the central level, expertise is generally available to evaluate the methodologic quality of the studies. This may not always be the case at the local level. Secondly, at the central level there is usually a prescribed process for presenting data and a set of methodological guidelines that need to be followed. Again, this may not always be
the case at the local level, although the Academy of Managed Care Pharmacy (AMCP) Format (McCain, 2001) is one attempt to introduce such a process.

The main implication of these differences is that, at the local level, there is much more concern over whether analyses can be trusted and whether there are potential biases in research sponsored by manufacturers. By contrast, at the central level, a company submission is, by definition, advocacy for the product and there is usually sufficient expertise available to undertake a detailed critical appraisal.

This suggests that, while elements of good practice in the conduct and reporting of PE/HE studies are relevant in all decision-making contexts, decision-makers’ needs for assistance in interpreting studies are greater at the local level.

3. DECISION-MAKERS’ ATTITUDES TO PE/HE STUDIES

There have been several surveys of decision-makers’ attitudes to PE/HE studies (see Table 1), plus a few general commentaries. For a study to be useful in a given decision, the decision-maker needs to be convinced of its reliability and relevance. A reliable study would be one giving accurate estimates that are free from bias. A relevant study would be one containing results that apply to the decision-maker’s own setting. (Some authors use the terms internal and external validity to refer to the same concepts.) The main findings, in relation to reliability and relevance, from the various surveys are summarized below.
### Table 1: Surveys of Decision-Makers’ Attitudes to PE/HE Studies

<table>
<thead>
<tr>
<th>Author (Date)</th>
<th>Country</th>
<th>Study Population</th>
<th>Survey Method</th>
<th>Headline Results</th>
</tr>
</thead>
<tbody>
<tr>
<td>Ross (1995)</td>
<td>Australia</td>
<td>Ministry officials (34)</td>
<td>Interviews</td>
<td>Main barriers to use of studies are: (i) short-term nature of the decision-making process; (ii) problems in interpreting studies; (iii) lack of timeliness in study results; (iv) importance of other factors in decision-making.</td>
</tr>
<tr>
<td>Luce and Brown (1995)</td>
<td>USA</td>
<td>Decision-makers from hospitals, HMOs and third-party payers (48)</td>
<td>Interviews</td>
<td>Range and sophistication of decision-making processes varies. Hospitals focus on traditional financial analysis, with the exception of pharmacy committees, which conduct socio-economic analyses. HMOs undertake outcomes assessments but exclude economics.</td>
</tr>
<tr>
<td>Luce et al (1996)</td>
<td>USA</td>
<td>51 MCOs</td>
<td>Telephone survey</td>
<td>Respondents rated clinical effectiveness and cost-effectiveness assessments more useful than quality of life assessments. Most plans were considering establishing a partnership with a drug company for disease management and would support some form of regulation of pharmacoeconomic claims.</td>
</tr>
<tr>
<td>Drummond et al (1997)</td>
<td>United Kingdom</td>
<td>Prescribing advisers (283), hospital directors of pharmacy (400), directors of public health (101)</td>
<td>Mail questionnaire</td>
<td>Use of studies was not extensive. Main obstacles were the inflexibilities in health care budgets and some concerns about the methodologic quality of studies.</td>
</tr>
<tr>
<td>Sloan et al (1997)</td>
<td>USA</td>
<td>Hospital directors of pharmacy (103)</td>
<td>Telephone survey</td>
<td>Cost-effectiveness was a minor tool in pharmaceutical decision-making. Reasons for not using CEA more often were: lack of information on the potential cost offsets; lack of independent sponsorship; inadequate expertise in economic evaluation.</td>
</tr>
<tr>
<td>Duthie et al (1999)</td>
<td>United Kingdom</td>
<td>17 pairs of NHS clinicians and managers</td>
<td>Interviews</td>
<td>A high proportion of statements conveying traditional health economics outcomes (eg, incremental ratios, QALYs) were either not understood or considered irrelevant.</td>
</tr>
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<td>Author (Date)</td>
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<td>Study Population</td>
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<td>Headline Results</td>
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<tr>
<td>Hoffmann <em>et al</em> (2000)</td>
<td>European Union countries</td>
<td>Government officials, healthcare managers, pharmacists, physicians (1041)</td>
<td>Mail questionnaires, interviews and focus groups</td>
<td>Studies are not widely used. Institutional problems, such as transferring budgets, and lack of credibility of studies, are important barriers. A better explanation of practical relevance of results and more training in health economics are needed.</td>
</tr>
<tr>
<td>Burns <em>et al</em> (2000)</td>
<td>United Kingdom and USA</td>
<td>Government officials, healthcare managers, purchasers (55)</td>
<td>Focus group</td>
<td>Barriers in the use of studies are: (i) overriding concern with cost rather than quality; (ii) difficulties in accessing clinical and cost-effectiveness data; (iii) insufficient training in interpretation and use of studies; (iv) lack of skills in translating evidence into practice.</td>
</tr>
<tr>
<td>Motheral <em>et al</em> (2000)</td>
<td>USA</td>
<td>Pharmacists or physicians working in health care organizations (241)</td>
<td>Mail questionnaire</td>
<td>Some respondents (90%) considering using PE information. 20% rarely or never act on this information while two-thirds occasionally do.</td>
</tr>
<tr>
<td>Cox <em>et al</em> (2000)</td>
<td>USA</td>
<td>Pharmacy benefit decision-makers (16)</td>
<td>Telephone interviews</td>
<td>Statements in terms of quality-adjusted life-years are difficult to conceptualize. High affinity toward disaggregated presentation of results.</td>
</tr>
<tr>
<td>Ginsberg <em>et al</em> (2000)</td>
<td>USA</td>
<td>Practising physicians (512)</td>
<td>Mail questionnaire</td>
<td>Most physicians accept that cost-effectiveness is important and appropriate in clinical practice, there is little uniformity in how cost-effectiveness decisions are implemented.</td>
</tr>
<tr>
<td>Anell and Svarvar (2000)</td>
<td>Sweden</td>
<td>Members of formulary committees (210)</td>
<td>Mail questionnaire</td>
<td>Respondents indicated an interest in economic evaluations, but warned that there was neither sufficient competence among formulary committee members, nor an adequate supply of relevant studies.</td>
</tr>
<tr>
<td>Hoffmann <em>et al</em> (2002)</td>
<td>United Kingdom</td>
<td>Decision-makers from 2 health authorities (12)</td>
<td>Focus group</td>
<td>General usefulness of studies recognized. However, value often limited because of the poor generalizability of results, the narrowness of research questions and lack of methodological rigour.</td>
</tr>
<tr>
<td>Grizzle <em>et al</em> (2000)</td>
<td>USA</td>
<td>Managed care decision-makers (31)</td>
<td>Telephone survey</td>
<td>Most managed care decision-makers believe pharmacoconomics information is important. The main barriers to using study results are: lack of relevance, drug silo mentality, lack of credible information, lack of resources, no focus on long-term costs and lack of expertise.</td>
</tr>
</tbody>
</table>
3.1 **Issues relating to reliability**

A major general concern of decision-makers is the lack of transparency in the reporting of PE/HE studies. This concern applies to all studies, but is probably greatest in the case of modelling studies, with which most healthcare decision-makers are less familiar. The lack of transparency also partly fuels decision-makers’ concerns about the potential sponsorship bias in PE/HE studies.

In addition to the relative lack of transparency in modelling studies, decision-makers are often concerned about the extensive use of assumptions and the extrapolation of benefits over a timescale not directly observed in the clinical trials themselves. For example, in one economic evaluation of cholesterol-lowering therapy (Caro *et al.*, 1998), only 10% of the benefit (in life extension) was observed during the trial itself. The remainder came from an extrapolation, over the lifetime of patients, from events (such as angina) observed during the trial follow-up period.

Whereas most economists would prefer the use of extrapolation, especially if this leads to the consideration of a more relevant time horizon or more relevant outcome, decision-makers tend to prefer the observed over the unobserved. The same is true of the analysis of practice patterns and treatment costs, where the estimates from physician expert panels are usually considered inferior to data from a clinical trial, patients’ charts, or an administrative database.

Finally, several of the surveys indicate that decision-makers are less comfortable than economists with the methods for calculating quality-adjusted life-years (QALYs) and
willingness-to-pay (WTP). First, they find the concepts behind these benefit measures a little difficult to understand. Secondly, they have some concerns about the reliability of the estimation methods themselves, and thirdly, in respect of QALYs, they have a more general concern about the aggregation of health benefits in a single index. Therefore, decision-makers often prefer to see the various components of benefit presented in a cost-consequences analysis.

3.2 Issues relating to relevance

Probably the main issue relating to relevance is that typically PE/HE studies do not explore budgetary impact. Whereas the cost-effectiveness ratio gives an indication of the value for money from a therapy, it says nothing about total cost. On the other hand, the decision-maker is often more concerned about affordability, which obviously depends on the overall volume of patients likely to benefit from the therapy and on whose budget the costs are likely to fall. Therefore, decision-makers often prefer to see various budgetary perspectives explored (along with the societal perspective), as well as an estimate of overall budgetary impact.

In the case of managed care in the USA, this may represent a real challenge, owing to the diversity of plans. However, it may be possible to develop a ‘reference case’ (Gold et al, 1996) for managed care, or to show budgetary impact, under different assumptions, for various timeframes (eg, 2 years, 5 years, etc).

A common justification for investments in higher cost therapies is that savings will be made elsewhere in the healthcare system (on other budgets), or in the future. Even
those decision-makers not adopting a ‘silo’ mentality (ie, concern only for their own
budget) sometimes doubt whether many of the savings will actually be achieved. Of
course, in many cases economists refer to freed resources rather than financial
savings. That is, the benefit from a shorter length of hospital stay is that the vacated
bed can be used in the treatment of another patient. Although decision-makers
understand these arguments, it is often difficult for them to take these on board when
living within a financial budget constraint. Indeed, a hospital or a health plan could
get into financial difficulties by adopting too many cost-effective interventions!

One way of bringing together the value-for-money and budgetary considerations
would be to explore the cost-effectiveness ratios (and budgetary impacts) of treating
different sub-groups of patients within the total patient population. (Sub-groups are
often defined by indication, or by various pre-treatment risk factors.) The current
consideration of these issues in PE/HE studies is, at best, patchy. However, it is also
controversial, as there may be insufficient patient numbers in clinical studies to
demonstrate differences between sub-groups of the overall patient population at the
conventional level of statistical significance.

Finally, some decision-makers doubt whether PE/HE studies undertaken in other
locations apply in their settings. There is a substantial literature on the transferability
of PE/HE studies and economists have developed methods to adapt study results from
one location to another (Drummond and Pang, 2001). However, the individual
decision-maker may still require a presentation of study results that reflects the local
situation. Often this can be dealt with by the use of sensitivity analysis or interactive
models.
4. ELEMENTS OF GOOD PRACTICE IN THE REPORTING OF PE/HE STUDIES

There are now a number of guidelines for the conduct and reporting of PE/HE studies. Some originate from decision-making bodies, in those jurisdictions where there is a formal requirement for cost-effectiveness evidence. Others originate from groups of academic researchers, or related groups interested in maintaining methodological standards in this field of research.

Several of the existing guidelines have recently been reviewed by Hjelmgren et al (2001). They concluded that, whilst there were differences among the published guidelines, there was some harmonization of methodological standards. The level of agreement on methodological aspects was higher for the formal guidelines than for informal guidelines, or general guidelines on health economic methods.

Not all the available guidelines for PE/HE studies specify a standard reporting framework or template. Details of 15 that do are given in Figure 1. It can be seen that there is a fair amount of agreement between the different guidelines in terms of reporting requirements. In the main they are aimed at increasing transparency (eg, state what comparator was used), although sometimes they embody elements of methodological prescription (eg, present the results with costs and effects discounted at 5%). Our analysis indicates that there is considerable agreement on what should be reported, even if the methodologic prescriptions differ slightly from guideline to guideline. (See the paper by Hjelmgren et al for more detail of the methodologic prescriptions.)
**Figure 1:** Reporting Requirements in 15 Published Economic Evaluation Guidelines

<table>
<thead>
<tr>
<th>Topic</th>
<th>Number of Guidelines Requiring a Report</th>
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<tbody>
<tr>
<td></td>
<td>0</td>
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<tr>
<td>Objective of study</td>
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<tr>
<td>Target audience</td>
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<tr>
<td>Viewpoint</td>
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<td>Comparator</td>
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<tr>
<td>Discounting</td>
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<tr>
<td>Uncertainty</td>
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<tr>
<td>Outcome measurement</td>
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<tr>
<td>Cost/resources measurement</td>
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<td>Time horizon</td>
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<tr>
<td>Modelling</td>
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<tr>
<td>Aggregated analysis</td>
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<tr>
<td>Form of evaluation</td>
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<tr>
<td>Incremental analysis</td>
<td></td>
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<tr>
<td>Costs/resources reported separately</td>
<td></td>
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<tr>
<td>Disaggregated analysis</td>
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</table>
4.1 **Relevance of other Task Force Reports**

Four of the other ISPOR Task Forces deal with aspects of study methodology; namely those on modelling, prospective studies, retrospective (database) studies and quality-of-life. All the reports of these Task Forces make methodological recommendations, many of which are carried forward as requirements for reporting of results. Therefore, we refer the reader to the reports of the relevant Task Forces, in particular any requirements for reporting.

4.2 **Additional reporting requirements for decision-makers**

Because of the concerns raised by decision-makers about the reliability and relevance of PE/HE studies, we propose some additional reporting requirements below. This represents an ideal list and we recognize that it may not be possible for the authors of PE/HE studies to address all these points in a published paper. However, serious consideration should be given to addressing them in formulary submissions and other direct communications to decision-makers.

(i) **Description of relevant patient population(s)**

The value for money of a given therapy depends on the patient population(s) in which it is used. The size of the patient population also affects budgetary impact. Therefore, the study report should clearly identify the relevant patient population(s) and, if possible, their size in the jurisdiction concerned.
(ii) **Budgetary perspectives and budget impact**

Decision-makers are interested in the cost of adopting the new therapy on their own budget and other budgets in their organization.

*Therefore, the study report should clearly identify the relevant budgets and the impact on each of adopting the new therapy.*

(iii) **Cost-consequences analysis**

Decision-makers appreciate a disaggregated presentation of the study costs and outcomes, prior to any aggregation in an incremental cost-effectiveness or cost-utility ratio.

*Therefore, the study report should include disaggregated costs and outcomes, comparing the new therapy with the existing one.*

(iv) **Costs, consequences and cost-effectiveness by patient sub-group**

Where there are relevant sub-groups of the patient populations, decision-makers are interested in how value-for-money varies by sub-group.

*Therefore, where relevant and feasible, the study report should present costs, consequences and incremental cost-effectiveness ratio by patient sub-group.*

(v) **Practical implications of adopting the new therapy**

Decision-makers sometimes find it difficult to understand the practical implications of adopting a therapy with a given incremental cost-effectiveness ratio. An alternative way of presenting results would be to explain what the adoption of the new therapy might mean in terms of budgetary impact and implications for the health of the relevant patient population(s). The analyst might also attempt to explain how and
when savings in the use of other healthcare resources may be achieved, although we recognize that much of this is context-specific.

Therefore, the study report should attempt to explain the impact, in practical terms, of adopting the new therapy.

(vi) **Listing of key assumptions and data sources**

A key concern of decision-makers is transparency in the reporting of PE/HE studies. At the local level in particular, decision-makers do not have the time or expertise to undertake detailed critical appraisals of studies.

Therefore, the study report should list all the key assumptions and data sources.

(vii) **Sensitivity analyses using the decision-maker’s own data and assumptions**

Economic data do not easily transfer from place-to-place and it is known that a number of factors are likely to affect the cost-effectiveness of healthcare interventions.

Therefore, the study report (or model) should facilitate sensitivity analyses, using the decision-maker’s own data and assumptions.

These reporting requirements are summarized in Table 3.
Table 3: **Additional Reporting Requirements for Decision-Makers**

1. The study report should clearly identify the relevant patient population(s) and, if possible, their size in the jurisdiction concerned.

2. The study report should clearly identify the relevant budgets, and the impact on each, of adopting the new therapy.

3. The study report should include disaggregated costs and outcomes, comparing the new therapy with the existing one.

4. Where relevant, the study report should present costs, consequences and incremental cost-effectiveness ratio by patient sub-group.

5. The study report should attempt to explain the impact, in practical terms, of adopting the new therapy.

6. The study report should list all the key assumptions and data sources.

7. The study report (or model) should facilitate sensitivity analyses using the decision-maker’s own data and assumptions.

5. **ISSUES FOR FURTHER RESEARCH**

In making these proposals we are conscious that there are may unanswered questions. These include the following:

- How do these recommendations fit with those of the other ISPOR Task Forces?

- How feasible is it for the manufacturers and sponsors of new technologies to be expected to meet these requirements; will the benefits to manufacturers justify the cost?
- Should these additional requirements be limited to submissions to individual decision-makers, or is it also realistic to address some of them in published papers?

- What scope exists for the harmonization of decision-makers’ requirements, so as to reduce the overall burden on manufacturers preparing submissions?

- What addition education do decision-makers at different levels need in the methods of PE/HE studies?

We have already begun thinking about some of these issues, but would welcome feedback from the ISPOR membership and from further afield.
REFERENCES


McCain J. System helps P&T committees get pharmacoeconomic data they need. *Managed Care* 2001: April: 24C-24J.


ACKNOWLEDGEMENTS

The Task Force is grateful to GlaxoSmithKline for an unrestricted grant to fund the review of published studies of decision-makers’ attitudes to PE/HE information. We are also grateful to François Schubert for his contribution to the work of the Task Force. Marco Barbieri is currently receiving financial support from Schering-Plough Inc for a fellowship at the Centre for Health Economics.

Finally, we are especially grateful to the healthcare decision-makers who have taken time to give us their views on PE/HE studies and how the practice of reporting studies can be improved.
APPENDIX

References for the 15 published guidelines considered in Figure 1.

Belgium:


Canada:


France:


Germany:


Hungary:

(To be added.)

Netherlands:

Norway:


Poland:


Portugal:


Switzerland:


United Kingdom:


USA:

