Irish Healthcare Technology Assessment Guidelines

Prepared by the National Centre for Pharmacoeconomics in Ireland

Contents

Irish Pharmacoeconomic Guidelines

1. Purpose

The purpose of the pharmacoeconomic guidelines is to provide the Department of Health, the GMS payments board, and prescribers, with information on the cost effectiveness of a health care technology[1]. This process will take place in the context defined by the agreement between the Irish Pharmaceutical Healthcare Association and the Department of Health.

It is important to note that "best value", or greater cost-effectiveness, is consistent with an increase in health impact achieved with an increase in the drug budget, as well as with the same health impact achieved with fewer resources. It follows that increasing the cost-effectiveness of Irish health technologies may or may not reduce health service expenditures, though it should result in greater health impact per unit of expenditure.

[1] The term "technology" includes any distinct practical method of delivering health care, including drugs; an alternative term is "health intervention".

2. General Approach

Following instruction from the Dept. of Health, it is envisaged that the first stage in the process of an economic evaluation would consist of a preliminary discussion between the Centre and representatives from the relevant pharmaceutical company, to determine information requirements.

When a formal submission of evidence is made, it is expected to weigh the benefit of a new technology against its costs in the Irish context and/or in a similar health service setting, using one of the approaches described in the appendix. It is important that the submission specifies, addresses, and answers a specific study question. This does not mean that data collection in Ireland (such as a randomised clinical trial) will necessarily be required; existing work, supplemented if possible by studies specific to Ireland, may be used to estimate the cost-effectiveness of the technology in question. Reasonable inference (by the standards of the medical and pharmaceutical literature) from populations studied elsewhere will be acceptable.

Any existing evaluation may be supplemented by new data; all data supplied by the pharmaceutical industry will be treated in a confidential manner. Finally, in the interest of fairness and transparency, companies will have access to the Centre's response to the submission, to discuss it, and if necessary, to appeal it. Evaluations will be carried out by the staff of the Centre and external consultants, under conditions which respect the necessary confidentiality requirements. Any requests for information from a third party will be brought to the attention of the relevant company.

3. The Guidelines

The requirements that should be considered may be described under the headings of study design, data analysis, and results presentation.

3.1 Study Design

3.1.1 Study Question

- The question being addressed should be clearly stated.
- The economic importance of the research question should be explained.
- The perspective adopted for the analysis (health care system, society) should be clearly stated and explained.

3.1.2 Selection of Alternatives

- The rationale for the particular comparison (of specific alternative treatments) should be given.
- The alternative treatments should be described in sufficient detail to enable the reader to assess their relevance.

3.1.3 Type of study

Generally cost effectiveness analysis (CEA) is appropriate. Cost utility analysis (CUA) should be applied when quality of life is the sole or major output of the therapy. If treatment options have been demonstrated to be equivalent, cost minimisation analysis (CMA) is appropriate. In certain circumstances, cost benefit analysis (CBA) is the best available means of clarifying the choices present (see appendix).

3.1.4 Benefit Measurement and Evaluation

- The primary outcome measure(s) for the economic evaluation should be clearly stated, such as cases detected, increased survival, or quality-adjusted life years (QALYs).
- If the measure involves the use of any psychometric instrument, such as a QALY or cognitive function questionnaire, its derivation, validation, and relevance should be briefly explained, and details given of the published evidence supporting it.
- If changes in production (an indirect benefit) are included, they should be reported separately, and their relevance to the study question discussed.

3.1.5 Method of Data Capture.

- High quality randomised trials, where possible, supplemented by information from other sources including meta analysis, observational data and modelling are acceptable. Other data sources and approaches should be discussed with the Centre before substantial resources are committed to them.

3.1.6 Costing

- Quantities of resources (drug amounts, or staff time) should be reported separately from the prices (unit costs) of those resources.
- Methods for the estimates of both quantities and prices (unit costs) should be given.

3.1.7 Irish Cost Data

- As yet, there are no agreed Irish cost models, such as those produced by the University of Kent in the United Kingdom, and much work needs to be done to generate valid Irish cost data. It follows that flexibility is required in applying the requirement that studies should consider the cost dimension in Irish terms. Where costs are applied from other contexts, there must be an explanation of the assumptions necessary to translate these costs to the Irish health system.

3.1.8 Modelling

- Details should be given of any modelling used in the study - for example, a decision tree model, epidemiology model, or regression model - especially the assumptions on which the model is based, and the methods used to obtain data, other than conventional data collection in a trial. The model and its assumptions should be justified.

3.1.9 Time Horizon

- The study period should be clearly described and appropriate to the disease and treatment. Long-term effects should be emphasised and a need for modelling discussed as above.

3.2 Data Analysis

3.2.1 Adjustments for the timing of costs and benefits

- The time period over which costs and benefits are measured should be given.
- The discount rate should be given, and the choice of rate(s) explained.
- If costs and benefits are not discounted, an explanation should be given.

3.2.2 Allowance for uncertainty

- When appropriate, details should be given of the statistical tests performed and the confidence intervals around the main variables.
- When a sensitivity analysis is performed, the choice of variables to be altered, and the ranges over which they were altered, should be justified.

3.3 Results Presentation

- Major outcomes - for example, treatment impact on quality of life - should if possible be presented in disaggregated as well as summary form, by patient groups.
- Any comparison with other health care interventions - for example, in terms of relative cost-effectiveness - should be made only when close similarity in study methods and settings can be demonstrated.
- The answer to the original study question should be given; any conclusions should follow clearly from the data reported, and should be accompanied by appropriate qualifications or reservations.

4. Conclusion

The fundamental test of all healthcare technology studies is: "what is a *reasonable* balancing of costs against health outcomes in the context given?". Important features of the "given context" include the inherent difficulties of measuring outcomes and costs, and the nature of the choices being investigated. The appropriate choice of evaluation approach is usually clear from the context, and from the existing literature.

It is envisaged that the guidelines will be reviewed and/or revised after a period of five years.

Appendix: Methods of Economic Evaluation Relevant to the Health Sector

A.1 Introduction

The weighing of alternatives against each other, in terms of their costs and benefits, so that choices can be clarified and rational decisions taken, is simple in principle. *Cost-Benefit Analysis (CBA)* evolved to deal with public sector decisions, and is regarded, in general, as the evaluation technique of choice. We briefly explain what *CBA* is, identifying its disadvantages in the health sector, and go on to consider *Cost-Effectiveness Analysis (CEA), Cost-Utility Analysis (CUA),* and *Cost-Minimisation Analysis (CMA)*, all of which weigh alternatives, in an attempt to achieve maximum health from limited resources.

A.2 Cost-Benefit Analysis

According to Sugden and Williams (1978), page 89:

"a cost-benefit analysis of a project requires the identification of all the effects of the project on the individual welfare of all members of the community. It then requires these effects to be measured in some common unit so that aggregate effects can be compared with aggregate costs."

When the CBA approach is applied to the health sector, it is necessary to set money values on health outcomes. There is a reluctance to value health in monetary terms [Sloan et al (1995), and Coast et al (1996)], and the approach has also been criticised [Coast et al (1996)] on the grounds that it will value more highly those interventions which are particularly desired or needed by those with higher wealth or income; this is regarded as unacceptable with regard to health care production.

However, in a particular situation, it may be true that the CBA approach is the best available means of clarifying the choices present; this is acceptable only if the valuation procedure is made explicit, and if the assumptions underlying the analysis are made clear. While there are not many examples of CBA in the health field, recent examples are Weisbrod (1995), and Ginsberg and Lev (1997).

A.3 Cost-Effectiveness Analysis

These considerations, and the political significance of health service efficiency in times of concern over budget levels, have led to the development of a branch of health economics, which has been called "extra-welfarist", in that it aims to maximise health per unit of currency spent.

If there exist two health programmes, m and n (for example, hip replacement and coronary artery bypass surgery), which are currently producing a certain number of procedures each, it will be possible to produce more in both programmes, to give additional (or marginal) benefits [MB_m, MB_n], for an additional (or marginal) cost [MC_m, MC_n]. If we have additional finance, the greatest impact on health will be achieved when it is allocated between the two interventions until:

$MB_m / MC_m = MB_n / MC_n$[2].

Cost-Effectiveness Analysis involves the comparison of the above ratios, for two or more health interventions, to determine which of them adds more to health per unit of expenditure, measuring health either by survival impact, or by the change in a health index. The approach has been widely used [Gyrdhansen et al (1998)], Whynes et al (1998), Badia (1997), and Freemantle (1994)], though it is not free from controversy,

particularly in respect of the costs which should be included [Johannesson and Metzler (1998), Brouwer et al (1997)].

A.4 Cost-utility analysis [CUA]

In a CEA, the impact of an intervention is assessed using (for example) survival changes, or numbers of cases of a disease avoided. A CUA measures the impact of an intervention on health-related quality of life using a utility-related measure, usually a Quality-Adjusted Life Year, or QALY. A number of such cardinal measures have been devised; they usually involve some method of comparing health states so that they can be located on a scale between zero (death or unconsciousness) at one end, and unity (full health) at the other. Interventions are thus compared in terms of the marginal cost per marginal QALY; the aim is to allocate resources until the number of QALYs gained is greatest, that is, until the marginal cost per marginal QALY is the same for all interventions. Quite apart from the problems of measuring cost this requires, the QALY approach, as a method for valuing interventions, remains controversial, because methods for valuing health states require a consensus about how valuations should be made, whose valuations should be used, and how the valuations of different individuals should be combined [Nord (1999)].

A.5 Cost minimisation analysis [CMA][3]

Here the interventions compared are shown (or are assumed) to be identical in their health impact. This identity is likely to be difficult to prove, though the assumption may make sense in a particular context; if it is true, or if the assumption is acceptable,

[2] This assumes that MBm and MBn are constant or falling as the numbers of m and n produced increase, and that MCm and MCn are constant or increasing, as the numbers of m and n produced increases. This is usually a realistic assumption.

the health impact per unit of expenditure will be maximised by allocating resources to the procedure, which has the lowest cost. In terms of our equation, above, when two procedures, A and B, are identical in terms of their health outcomes, the allocation of resources will have its greatest impact on health when adjustments to the production levels of A and B are made until:

$MC_A = MC_B$.

Having established that the health outcomes from the two procedures were indeed the same, a CMA would compare all the costs differentiating the two procedures, to determine which delivered the common outcome for the least cost.

Cost Minimisation Analysis should only be used where there is evidence showing that the outcomes are the same. If this evidence is not available then focusing on costs alone, to the neglect of benefits, could result in sub-optimal treatment of patients.

[3] In the United States, CMA is known as Cost-Effectiveness Analysis, and our definition of CEA does not appear in US health economics texts [Santerre et al (1996), Folland et al (1993)]

List of References

Alban A, Gyldmark M, Pedersen AV, Sogaard J The Danish Approach to Standards for Economic Evaluation Methodologies *Pharmacoeconomics* 1997 12(6) 626-636

Bootman L, Townsend R, McGrath W *Principles of Pharmacoeconomics* Cincinnati, Ohio: Harvey Whitney Books 1996

Buxton MJ, Drummond MF, van Hout BA et al Modelling in Economic Evaluations: An Unavoidable Fact of Life *Health Economics* 1997 6(3) 217-228

Canadian Co-ordinating Office for Health Technology Assessment *Guidelines for Economic Evaluation of Pharmaceuticals: Canada* 2nd edition Ottawa: CCOHTA, 1997

Canadian Co-ordinating Office for Health Technology Assessment *A Guidance Document for the Costing Process* Ottawa: CCOHTA, 1996

Carlin PS, Sandy R Estimating the implicit value of a young child's life *Southern Economic Journal* 58 pp 186-202 1991

Coast J, Donovan J, Frankel S *Priority Setting: The Health Care Debate* Chichester: Wiley 1996

Commonwealth Department of Human Services and Health (CDHSH) *Guidelines for the Pharmaceutical Industry on the Preparation of Submissions to the Pharmaceutical Benefits Advisory Committee* Canberra: Australian Government Publishing Service 1995

Commonwealth Department of Human Services and Health (CDHSH) *Background Document (on the use of economic analysis as a basis for inclusion of pharmaceutical products on the Pharmaceutical Benefits Scheme)* Canberra: Australian Government Publishing Service 1993

Culyer AJ The normative economics of health care finance and provision *Oxford Review of Economic Policy* 1989 5 pp 34-58

Drummond MF, Stoddart GI, Torrance GW*Methods for the economic evaluation of health care programmes* 2nd ed. New York: Oxford University Press 1997

Folland S, Goodman AC, Stano M *The Economics of Health and Health Care* New York: Macmillan 1993

Freemantle N, House A, Song F, et al Prescribing selective serotonin reuptake inhibitors as strategy for prevention of suicide *British Medical Journal* 1994 309 pp249-253

Garattini L, Grilli R, Scopelliti D, Mantovani L A Proposal for Italian Guidelines in Pharmacoeconomics *Pharmacoeconomics* 1995 7(1) 1-6

Ginsberg GM, Lev B Cost-Benefit Analysis of Riluzole for the Treatment of Amyotrophic Lateral Sclerosis *Pharmacoeconomics* 1997 12(5) 578-584

Gorham P Cost-Effectiveness Guidelines - the Experience of Australian Manufacturers *Pharmacoeconomics* 1995 8(5) 369-373

Gyrd-Hansen D, Sogaard J, Kronberg O Colorectal Cancer Screening: Efficiency and Effectiveness *Health Economics* 7(1) pp 9-20 1998

Henry D Economic Analysis as an Aid to Subsidisation Decisions: the Development of Australian Guidelines for Pharmaceuticals *Pharmacoeconomics* 1992 (1) 54-67

Hillner B, Hollenberg JP, Pauker SP Postmenopausal estrogens in prevention of osteoporosis *American Journal of Medicine* 1986 80 1115-1127

Jacobs P, Bachynsky J, Baladi J-F A Comprehensive Review of Pharmacoeconomic Guidelines *Pharmacoeconomics* 1995 8(3) 182-189

Johannesson M, Meltzer D Some Reflections on Cost-Effectiveness Analysis *Health Economics* 1998 7(1) pp 1-7

Nord E *Cost-Value Analysis in Health Care; Making Sense Out Of QALYs* Cambridge University Press 1999

Rovira J, Antonanzas F Economic Analysis of Health Technologies and Programmes: A Spanish Proposal for Methodological Standardisation *Pharmacoeconomics* 1995 8(3) 245-252

Santerre RE, Neun SP *Health Economics: Theories, Insights, and Industry Studies* Chicago: Irwin 1996

Sugden R, Williams A *The principles of practical cost-benefit analysis* Oxford: Oxford University Press

Weinstein MC Estrogen use in postmenopausal women - costs, risks, benefits *New England Journal of Medicine* 1980 303 308-316

Weisbrod BA Economics of mental illness: costs, benefits, and incentives , chapter 2 of *Health Economics of Depression,* B Ronsson, J Rosenbaum, Chichester: Wiley

Whynes DK, Neilson AR, Walker AR, Hardcastle JD Faecal Occult Blood Screening for Colorectal Cancer: Is It Cost-Effective? *Health Economics* 7(1) pp 21-30 1998

Zethraeus N Willingness to pay for hormone replacement therapy *Health Economics* 7(1) 1998 pp31-38