Decision trees and prescriber choices

Alan Stewart

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ALAN STEWART

All correspondence to:

Alan Stewart
PSSRU
University of Kent at Canterbury
Canterbury
Kent CT2 7NF

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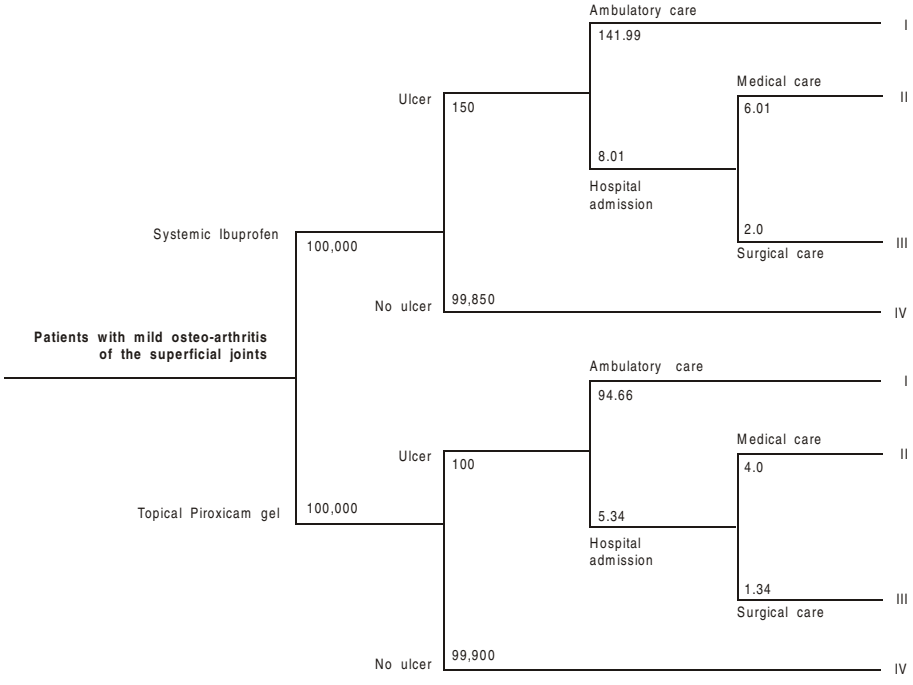
Introduction

Prescriber decisions are increasingly being pressured by a supply of economic information in the form of cost-effectiveness studies, or similar evaluations, of a range of pharmaceuticals. These have been welcomed by Virginia Bottomley and a set of guidelines for these studies has been drawn up by the Department of Health and the pharmaceutical industry[1].

Such economic studies are now being published in a wide range of journals and are increasingly being used in promotional literature and publicity handouts by the pharmaceutical companies. Many of these studies are using decision trees with which to represent clinical problems and perform analysis of the costs of treatment. One recent example compared alternative NSAIDS in the treatment of osteoarthritis[2].

Figure 1



The study that constructed the above diagram tells the reader that there is "an expected cost for three months treatment of £89.12 for oral ibuprofen and £54.57 for topical piroxicam gel".

Such answers raise the question of what it is that they actually mean and what they can offer to a clinician. They may appear to be a marketing ploy, but they do offer potential to be used positively by clinicians, pharmacists and others involved in pharmaceutical prescribing or purchasing.

Decision Analysis

These studies are based on the principles of decision analysis and represent given clinical problems using a fairly standard diagrammatic representation and notation. In Figure 1 the lines represent paths through standard treatment options. The points where a clinician makes a choice between alternatives are represented by an open rectangle. Where there is uncertainty as to the outcome of an event, such as a treatment involving a possibility of success or failure, this is represented by an open circle. The end point of the analysis is shown by a shaded circle. They lay out a defined and structured set of events that may occur in a defined sequence. This structure incorporates a number of probabilities at various points, defined as chance nodes, which generate cumulative chances of arriving at any particular end point in the diagram via a particular discrete path through the defined set of events. These techniques are not just used in economic analysis, they have also been used in pure clinical decision making[3]. The tree itself is just a flow diagram that visually represents decisions and outcomes in a set order.

Data Sources

But where does all the data come from for this analysis? One source that is often used is existing results from clinical trials, where decision analysis techniques can be used to generate retrospective economic evaluations. This has been done recently in some studies evaluating antidepressants in the context of the current debate over using SSRIs or TCAs[4,5]Alternatively, data is often `synthesised' by evaluating a range of existing pieces of clinical research though a process of literature search and review that may be extended to a

formal meta-analysis. This is a valuable procedure, but one that is subject to a number of possible biases: if followed it should be performed rigorously and with regard to principles laid down in the existing literature[6, 7]. It is important that the clinical data used is credible and rigorously constructed. If there is any doubt about this, the conclusions of economic studies may be strongly criticised, as shown in a recent exchange of views on one of the antidepressant evaluations referred to earlier[8,9].

Evaluation and outputs

Moving on from this set of probabilities, the carrying out of cost analysis and ultimately cost-effectiveness analysis is the next stage in the evaluation. While these models calculate cumulative probabilities along their paths they also aggregate the costs incurred at each stage of the process. Thus each end point has an accumulated probability of a patient arriving at that discrete point and also an aggregate expected cost for the health care resources utilised along the way. If one then multiplies each of these aggregate expected costs by the associated probability, the result is a weighted cost for that particular point: the sum of these weighted costs is the average expected cost of any patient being entered into the analysis at the first point in the tree.

The above process may sound complex or arithmetically tortuous, but in most studies the actual calculation of expected costs will be performed by one of a number of software packages available in the market. The analyst's role is in defining the problem and setting up the structure for analysis, then supplying the inputs of costs and clinical data.

This process therefore generates an average expected cost for all patients starting a course of treatment. For a clinician seeking to understand the economic implications of prescribing decisions, this means that for each patient entering therapy, using the selected treatment, the expected cost will equal the given figure. Individual patients will vary widely in terms of their clinical progress and hence their consumption of resources and resulting costs. This is

reflected in the decision tree by their completion at different end points. But these variations are accounted for in the tree by the use of probabilities providing a weighting to each aggregate set of costs. Ideally, a decision analysis paper should present not just this calculated expected cost, but also the actual costs that will be incurred if patients experience specific sets of clinical outcomes. For example, the paper could list costs for those who are "cured" by the drug without any side-effects or need for repeated courses and for those at the other extreme, who do not respond and hence need other treatments.

Therefore the end result, the expected cost, is what we expect to happen if a specific treatment is chosen and offered as therapy to a representative sample of patients. By repeating the analysis for different drugs or therapeutic options, with differing sets of probabilities, we can evaluate the cost implications of switching from the existing option to one of a range of alternative therapies.

Testing the results

These figures should however, be tested and evaluated by performing thorough sensitivity analysis. During this process the assumptions in the analysis are tested by systematically varying the values used for key elements in the study which may be sensitive to change. This area has recently been reviewed[10] and some key aspects of its role identified. For example, one can test for the uncertainty relating to variability in sample data, such as the actual package of resources that are used in providing care. Not all clinicians will treat patients in the same manner: sensitivity analysis can allow comparisons of costs where different quantities of specific items are provided. Or the costs of providing care within a given facility may well vary according to the intensity with which the capacity is used. The adjustments allowed in sensitivity analysis allow a range of results to be generated that will cover a range of circumstances.

There may also be uncertainty as to the evidence used for efficacy: are the results obtained in

a clinical trial setting generalisable to clinical practice? By using sensitivity analysis, one can examine the impact of efficacy at levels that clinicians would expect to see in everyday practice.

Some analyses may go beyond the calculation of costs or cost-effectiveness ratios and introduce ideas of the quality of life, performing what is called cost-utility analysis[11]. The general principles of criticism described here apply to these studies as well, but they also raise further issues on quality of life measurement which are dealt with in the specialist literature[12,13].

Summary

But when the analysis produces an expected cost, what does that figure actually represent? It is an estimate of the average cost that will fall on the health services, per patient treated, if a very large number of patients are treated following the treatment pattern suggested in the study. As shown in the decision tree various sub-groups of patients will incur different figures for total costs. But over the whole range of patients, the high and low cost outcomes will balance out to give the average figure, which is the expected cost. Clinicians can compare these figures as part of the process of considering whether they really should switch between drugs on the basis of differing prices. It may well be that giving in to pressures to switch to lower-price drugs will not actually help health care budgets, regardless of the impact on patient well-being.

So what are the key points to remember when evaluating these decision analysis studies?

i. Does the treatment path represent a credible set of events and decisions i.e. can clinicians recognise the decision tree as representing procedures that approximate to their own practices?

ii. Is the data used in the study credible and valid for this particular piece of analysis?

iii. If it comes from a clinical trial the usual discretion must be exercised before accepting the validity of the toleration, efficacy and adverse events figures.

iv. If the data is synthesised from available literature, have rigorous meta-analysis procedures been followed? Or is the literature search and review guilty of biases, such as careful selection of papers to be used or of `footnote chasing' where the use of references from papers known to the author shapes the final set of papers incorporated into the analysis.

All the above areas have to be examined by the reader of any economic study presenting a decision tree. If the study is accepted as having dealt with these points then it may be useful as a way for clinicians to move towards a more optimal allocation of their prescribing budgets. If the reader is unsatisfied with the rigour of the study's design, methodology and analysis, then its conclusions should be regarded with the same caution and suspicion that would be attached to a clinical trial report that did not appear to be methodologically rigorous.

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