Aiding clinical decisions with decision analysis

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As clinical decision making gets ever more complex, new analytical approaches are being developed to help. Decision analysis is used to structure complex decision problems in an uncertain environment by systematically linking decision choices with expected outcomes. Such models can include the probabilities of outcomes, patient preferences and costs. These models can help to advise about therapeutic avenues. This paper examines the nature of decision analysis, and explores the pitfalls that arise in interpreting the findings from published studies.

Clinicians face many complex decisions in diagnosing and treating patients. Most diagnostic procedures provide not certainty but probabilistic information about the likelihood of disease. Treatments likewise may change the expected frequency of outcomes (desired or unwanted) but rarely offer guaranteed results. In addition, patients may have strong (but not always expressed) preferences regarding treatment strategies as well as their resultant outcomes. These preferences could and should be taken into account. It is under such manifold difficulties that doctors and their patients must make choices about diagnostic and treatment strategies.

Clinical research provides information that can feed into decisions about treatment strategies. Epidemiological follow-up studies can tell what happens to patients in the long term. Trial data can show the difference that interventions can make on a range of health outcomes. Patient preference studies can establish the utilities (values) that patients attach to certain health states. Combining and integrating information from all of these studies can help answer the question ‘which treatment strategy is most likely to bring the most benefit to the patient?’

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Often, integrating information from research studies into clinical decision making is an informal process carried out by doctor and patient during a consultation. Practitioners of evidence-based medicine may be more explicit in quantifying risks and benefits (Sackett et al, 1997) but this usually relates to clarifying the impact of a single intervention (Davies, 1998a,b; Davies and Crombie, 1998). However, for more complex decision problems, explicit analytical methods (called decision analyses) are available to assist.

Decision analysis (DA) is an approach to structuring multi-layered problems and analysing the likely benefits and costs from making certain decision choices. Investigations of clinical decisions using the techniques are beginning to appear in print, and such analyses can be used to underpin clinical guidelines.

This paper explains the rationale for a DA approach and explores the main features of the technique. We highlight the advantages of the approach and expose some of the common pitfalls that may arise in using and interpreting the findings.

WHEN IS DA APPROPRIATE?

DA may be appropriate when three factors come together: complexity, uncertainty and imperfect information. Each of these is explored below. Crucially, in many clinical decision problems it is the presence of all three of the factors which so clouds the decision choice.

Complexity

Technological and pharmacological advances in recent decades have presented clinicians with a far greater range of diagnostic and treatment options than hitherto. Clinical decisions may not be limited to a simple ‘this or that’ choice. They may be linked in chains where a decision at one point simply leads to another series of choices further down the line.

For example, consider a patient with a hip prosthesis complaining of pain and with signs of infection. Should the patient be treated medically or surgically? If surgically, should the prosthetic replacement be done in a one-stage or a two-stage procedure? What use then of antibiotics? Which drug or combinations and by what route of administration? With this level of complexity, it may be difficult to assess the ideal course of action.

A second problem is the potential for trade-offs between different outcomes. Patients may have divergent preferences in how they trade off gains in one outcome against losses in another. For example, the choice between radiotherapy and surgery for throat cancer may involve sacrificing life expectancy for increased quality of life. Thus, making a decision requires striking a balance between various desirable outcomes and the unpleasantness associated with some treatments, with the knowledge that for some decisions there can be no going back (e.g. in the case of surgery).

Uncertainty
In medicine the relationships between actions and outcomes are usually probabilistic rather than deterministic. There is always a chance that a good decision may turn into a poor outcome (or vice versa) even if the problem is well understood. Thus any analysis of a decision needs to manipulate probabilities rather than certainties. Such manipulations may be non-intuitive. Further, some individuals may be more 'risk averse' (disinclined to take chances) than others. Thus the attitude of the participants to risk have to be taken into account in order to arrive at an optimal decision.

Imperfect information
For all the burgeoning clinical research base, much remains unclear about the full implications of treatment decisions. The links between interventions and the complete range of outcomes may not have been established in all patient groups. In addition, the doctor may know little about patient preferences, either for outcomes (how do they value health improvements? how do they feel about side effects?) or processes (how averse to surgery are they? do they value keeping their options open?). Thus decisions must be made with only an incomplete picture. Furthermore, even in the unusual situation where all the necessary information is available, there remains the problem that the human mind may not be able to integrate effectively such complex information.

THE DA APPROACH

Many clinical decisions are not supported by clear evidence such as a randomized control trial directly comparing the treatment options. Nonetheless decisions still have to be made. DA is an attempt to assist in this process. It is an approach to structuring a decision problem so that decisions are related to outcomes in an explicit manner.

DA incorporates and helps identify sources of uncertainty, and presents this uncertainty in a quantitative way using probabilities. Once the problem is structured and tagged with data, it then becomes possible to analyse the expected outcomes (and, if necessary, costs also) for any given set of decision choices.

There are five basic steps to a DA (Clemen, 1996; Drummond et al, 1994; Petitti, 1994):

Identifying and bounding the problem
This involves identifying the set of decisions under study, and listing the full range of possible outcomes. By carefully considering all aspects of the problem (including the aims and preferences of patients and health professionals), alternatives that were not so obvious at the outset (such as 'do nothing') might be uncovered and considered explicitly.

Structuring the problem
The aim is to simplify the problem by breaking it down into its component parts. The full range of outcomes which may flow from any decision are identified, together with any further decisions which may then need to be taken. The aim is to develop a tree structure that flows from decisions (branch points) along various routes to different outcomes. The resulting structure provides clear linkages between decisions and outcomes. These are called decision trees, and an example is shown in *Figure 1*.

Adding information
A decision tree is a representation of the decision problem. However, before the tree can be analysed, it needs to be completed by adding in some quantitative information. These are the probabilities of the various outcomes at each stage of treatment, and the values that patients attach to these outcomes. Such information may come from a variety of sources, such as randomized control trials, observational studies, meta-analyses and reviews, new primary data collection and/or expert consensus.

Analysis of expected benefits
Analysis is done by a method of 'folding back and averaging'. That is, the 'expected value' of each decision choice is calculated from the probabilities assigned to the pathways and the utilities attached to the outcomes reached by those routes. This is done to determine the best strategy for the problem in hand, for example to identify the route to the likely best outcome, or to the best cost-utility ratio depending on the stakeholders' objectives.

Sensitivity analysis
The complexity involved in decision trees may mean that small shifts in the individual probability estimates, or in the values applied to outcomes, can lead to large changes in the likely

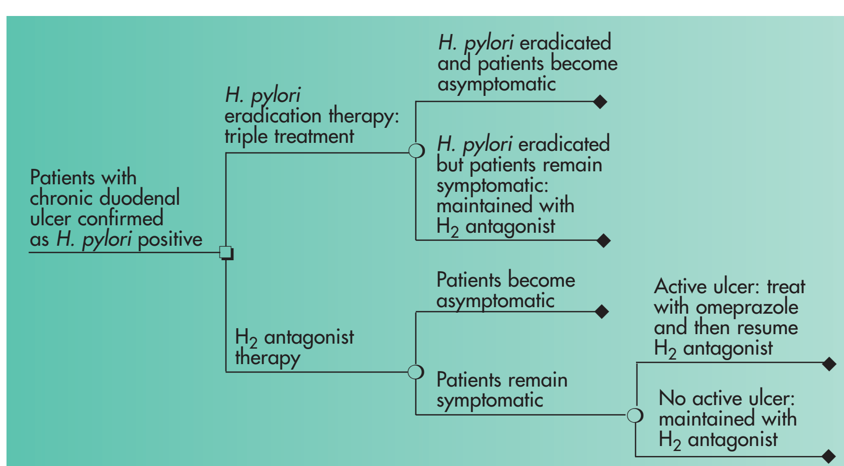

Figure 1. An example of a decision tree. This decision tree outlines a key decision faced in managing patients with chronic duodenal ulcer who are known to be infected with Helicobacter pylori*: should eradication therapy be tried before use of H_2-antagonist therapy? At the various branch points, ❒ represents an explicit treatment choice; whereas ❍ reflects a range of possible outcomes arising by chance from that choice. ◆symbols represent each possible final health status for a patient.*

optimal decision. Sensitivity analysis is the process of repeatedly analysing the tree using different values for probability and utility variables. The robustness of the conclusions from the model can then be assessed on the basis of variations in the expected values.

DA AS AN AID TO DECISION MAKING

Clinicians cannot be expected to develop formal decision models when faced with making individual patient decisions. Yet DA models are appearing more frequently now in the literature and these can be used to inform day-to-day clinical practice. If this is so, however, then some grasp of the relative strengths and weaknesses of DA techniques is necessary so that clinicians can be discerning consumers of the information offered.

DA techniques can be used to guide the management of individual patients or can be used to address policy questions about the use of treatment for groups of patients (Lilford et al, 1998). At present few models are available that can be customized to individual patient decisions (although the technology is available). More usually, DA models are used to clarify which treatment options are best on aggregate for patients with a given set of characteristics.

DA offers a number of advantages over more informal and intuitive attempts at synthesizing a mass of complex data. Foremost among these are:

Clarity and quantification

DA can clarify the extent of a decision problem through a thorough, logical and quantitative evaluation of alternative strategies. It makes the linkages between actions and outcomes (both good and bad) clear and explicit.

Consideration of preferences and costs

Stakeholders' objectives and preferences can be modelled explicitly in DA models. Costs can also be included if desired.

Explicit trade-offs

In some randomized trials the optimal decision remains uncertain because there may be several outcomes — and although the intervention may reduce the risk of one event it may increase the risk of others. DA can incorporate these trade offs, taking into account the patient characteristics which lead to different levels of risk (Naglie and Detsky, 1992; Lilford et al, 1998).

Exploration of options

DA can facilitate exploration of a series of 'What if...?' questions to allow a deeper understanding of the interconnectedness of decisions and outcomes.

Openness

In DA, the problem is defined explicitly. This allows critics to identify parts of the model or underlying assumptions with which they disagree. This fosters clear communication and rational debate as to the model validity, and in turn may bring about better conceptualizations and, ultimately, better decision-making.

PITFALLS IN DA

Like any research technique DA is not without its limitations. A good DA may help inform clinical decisions but it cannot replace human input. In particular, any DA model is only as good as the data on which it is based. A number of potential pitfalls are described below together with suggestions for their avoidance.

The boundaries of the problem may miss important features

One of the aims of DA is to focus the problem. Consequently the problem definition might omit important decision options (for example 'watchful waiting') or may fail to consider all possible outcomes (for example rare but disastrous side-effects). However, the explicit nature of DA leaves such omissions open to scrutiny, and the question is not whether any particular DA is truly complete but whether its coverage makes it appropriate for the decision under examination.

Probabilities of different outcomes are only estimates

These probabilities may be derived from either published literature or may simply be best guesses. As such, there may be considerable doubt as to the accuracy of these estimates. Sensitivity analysis can help to assess whether the uncertainty surrounding these estimates is important. If the model findings are consistent, despite variations in the data estimates, then the findings are said to be robust.

Expected benefits are benefits on average

The purported benefits from any decision are expected benefits in the statistical sense only. That is, any individual patient may benefit completely or not at all. Expected benefits could be the result of a small gain for everyone or a mixture of large gains for some and losses for others. That is, aggregation inevitably averages out the experiences of both winners and losers, and DAs yield only this average value.

Patient preferences are usually in aggregate

Although it would be possible to tailor a DA model to an individual using his/her own personal preferences, in practice this is problematic and rarely done. The patient preferences included are usually only incorporated in aggregate. Thus the recommendations from DA models may mislead if any individual patient has very different preferences from the group mean (for example, a very strong aversion to side-effects may override any expected benefits from therapy).

Estimating utilities for patient preferences is inexact and contentious

Various methods are used to measure utility but none is fully established. The major problem is that different techniques can give diverse and at times conflicting results. Further, individual preferences may vary with time or experience, which may not be reflected in the analysis (Krahn et al, 1994). The most popular methods of measuring utility are Standard Gamble (SG) (Feeny and Torrance, 1989; Torrance, 1987), Health Utility Index (HUI) (Boyle et al, 1995; Torrance et al, 1996), and Time-Trade-Off (TTO) (Torrance et al, 1972; Torrance, 1987). This part of any DA model may need special examination and assessment. However, the standard gamble technique is generally considered to be the most reliable method of utility measurement (Feeny and Torrance, 1989; Torrance, 1987). It is based on expected utility theory, and it is the only method with an underlying theoretical base and hence it could be argued that this should be the gold standard in the setting of DA.

DA models tend to focus on health outcomes and ignore process utilities

Patients may gain benefits simply from having treatments even when no good outcomes accrue. For example, participants in assisted conception programmes may value the experience even if they do not conceive. Conversely, patients may fear and dislike surgery even when it produces good outcomes; and other treatment avenues may make considerable demands on patients in terms of clinic visits or monitoring. Thus patients may gain or lose utility from factors other than simply their health outcomes — and not all DA models take these factors into account.

Finally, patients (and doctors) may value the ability to keep treatment strategies under review — whereas certain decisions preclude this possibility (a decision to proceed with surgery offers no possibilities of going back, while drug therapies may offer more flexibility). Because DA focuses on net expected values based on health states, it does not usually take into account the utility gained or lost in the processes of care.

Measurement of benefits and costs may contain hidden assumptions

DA models which look at benefit/cost ratios may use differing assumptions in estimating either or both of these two quantities. Exploration of these assumptions is needed to reassure that they are reasonable. Two previous papers in this series provide some advice (Neilson and Davies, 1998, 1999).

CONCLUSIONS

DA offers a formal and structured approach to integrating large amounts of complex and probabilistic data. As such it can clarify decision problems, quantify (on aggregate) the benefits or otherwise of certain decisions, and incorporate both costs and patient preferences. Good DA models do this in an open and explicit way, using sensitivity analyses to explore the robustness of their recommendations.

Nonetheless, DA models are complex to construct, contain many assumptions and make heavy demands on empirical data. Because of these limitations, DA models require careful appraisal and remain simply an aid to decision-making, not a substitute for human judgment. HM

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KEY POINTS

- Clinical decision-making is hampered by the complexity of the decision problems, the inherent uncertainty in the links between interventions and outcomes, and the incompleteness of the data available to inform choices.
- Decision analysis models structure decision problems using decision trees and thus link choices to expected outcomes.
- Decision analysis models offer advantages over informal assimilation of data in that they are open, explicit, structured, logical and quantitative.
- Decision analysis models can be extended by including patient preferences (utilities) attached to potential health outcomes. They can also include costs.
- Decision analysis models may still mislead if they are conceptually incomplete or are based on poor empirical data.
- Decision analysis models provide recommendations based on the aggregation of expected costs and benefit, whereas actual clinical decisions are applied to individuals. Thus care is needed when particularizing the findings from decision models to the care of individuals.
- Good sensitivity analyses can do much to bolster confidence in the robustness of the recommendations that emerge from decision analysis models.