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Elements of decision making in health care

And take the case of a man who is ill. I call two physicians: they differ in opinion. I am not to lie down and die between them: I must do something.

Samuel Johnson

1.1 Introduction

How are decisions made in practice, and can we improve the process? Decisions in health care can be particularly awkward, involving a complex web of diagnostic and therapeutic uncertainties, patient preferences and values, and costs. It is not surprising that there is often considerable disagreement about the best course of action. One of the authors of this book tells the following story (1):

> Being a cardiovascular radiologist, I regularly attend the vascular rounds at the University Hospital. It's an interesting conference: the Professor of Vascular Surgery really loves academic discussions and each case gets a lot of attention. The conference goes on for hours. The clinical fellows complain, of course, and it sure keeps me from my regular work. But it's one of the few conferences that I attend where there is a real discussion of the risks, benefits, and costs of the management options. Even patient preferences are sometimes (albeit rarely) considered.
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> And yet, I find there is something disturbing about the conference. The discussions always seem to go along the same lines. Doctor R. advocates treatment *X* because he recently read a paper that reported wonderful results; Doctor S. counters that treatment *X* has a substantial risk associated with it, as was shown in another paper published last year in the world's highest-ranking journal in the field; and Doctor T. says that given the current limited health-care budget maybe we should consider a less expensive alternative or no treatment at all. They talk around in circles for ten to 15 minutes, each doctor reiterating his or her opinion. The professor, realizing that his fellows are getting irritated, finally stops the discussion. Practical chores are waiting; there are patients to be cared for. And so the professor concludes: 'All right. We will offer the patient treatment *X*.' About 30% of those involved in the decision-making process nod their heads in agreement; another 30% start bringing up objections which get stifled quickly by the fellows who *really* do not want an encore, and the remaining 40% are either too tired or too flabbergasted to respond, or are more concerned about another objective, namely their job security.

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The authors of this book are all familiar with conferences like this. We suspect our readers also recognize the scenario and that they too have wondered, 'Isn't there a better way to make clinical decisions? Isn't there a better way for health professionals, policy makers, patients, and the general public to communicate with each other and talk things out when the going gets tough?'

This book addresses these questions. The methods we present can be helpful to all decision makers in the health-care arena – patients; physicians, nurses, other providers of clinical services; public health and hospital administrators; health-care payers in both the private and public sectors; and clinical and public health researchers whose job it is to offer wise and reasoned counsel.

Health-care decisions have become complex. As recently as a century ago, a physician had only a narrow range of possible diagnoses, a handful of simple tests, and a few, mostly ineffective, treatments to choose from. For example, the first edition of the justly famous *Merck Manual* (1899) ran to 192 pages. Since then our understanding of disease processes and our ability to control them have vastly increased, but so too has the complexity of health-care decisions. The 1999 centennial edition of the *Merck Manual* runs to 2833 pages (2). Currently our health-care technologies are expanding even further and faster, as is our knowledge about them, making modern electronic media indispensable in providing up-to-date information. Websites and mobile applications summarizing the evidence have proliferated over the last decade. All this knowledge needs to be integrated in a logical and wise fashion in order to optimize the decisions we make.

While new treatments have improved the outcome for many conditions, and even eliminated some diseases such as smallpox, many treatments are 'halfway' technologies that improve a condition but do not cure. For example, in cancer, there are many new, useful but sometimes taxing treatments that improve the prognosis without curing. Along with this increase in management options, we now contemplate treatment in a broader range of diseases, from mild hypertension to major disfigurement. This combination of a broad range of illnesses and imperfect treatment options increases our potential to help, but it also increases costs and makes decision making more complex and difficult. In this chapter, we outline a systematic approach to describing and analyzing decision problems. This approach, decision analysis, is intended to improve the quality of decisions and of communication between physicians, patients, and other health-care professionals. Decision analysis is designed to deal with choice under uncertainty and so it is naturally suited to both clinical and public health settings. We believe that decision analysis is a valuable tool for physicians and others concerned with health-care decision making, both for decisions affecting individual patients and for health policy decisions affecting populations of patients. The ability of physicians collectively to command a

vast array of powerful and expensive diagnostic and therapeutic interventions carries with it a social responsibility to use these resources wisely. Decision analysis is a systematic, explicit, quantitative way of making decisions in health care that can, we believe, lead to both enhanced communication about clinical controversies and better decisions. At a minimum, the methods we expound can illuminate what we disagree about and where better data or clearer goals are needed. At best, they may assure us that the decisions we make are the logical consequences of the evidence and values that were the inputs to the decision. That is no small achievement.

1.2 Decision making and uncertainty

Unlike most daily decisions, many health-care decisions have substantial consequences and involve important uncertainties and trade-offs. The uncertainties may be about the diagnosis, the accuracy of available diagnostic tests, the natural history of the disease, the effects of treatment in an individual patient or the effects of an intervention in a group or population as a whole. With such complex decisions, it can be difficult to comprehend all options 'in our heads,' let alone to compare them. We need to have some visual or written aids. Hence a major purpose of decision analysis is to assist in comprehension of the problem and to give us insight into what variables or features of the problem should have a major impact on our decision. It does this by allowing and encouraging the decision maker to divide the logical structure of a decision problem into its components so that they can be analyzed individually and then to recombine them systematically so as to suggest a decision. Here are two representative situations that can be addressed with this approach:

EXAMPLE 1

As a member of the State Committee for common childhood diseases, you have been asked to help formulate a policy on the management of chronic otitis media with effusions (also known as 'glue ear'). Glue ear is the most common cause of hearing problems in childhood and can lead to delayed language development. Many treatment choices exist, including grommets (pressure-equalizing tympanostomy tubes), analgesics, antibiotics, vaccinations (pneumococcal and influenza) and hearing aids (3). However, since glue ear usually resolves spontaneously, you might also choose to do nothing, at least initially. Given these various treatment options, should your committee recommend monitoring for hearing loss, treatment with grommet insertion, or the use of hearing aids? For example, tympanometry, which measures the eardrum's ability to move, can be used as a monitoring tool, though an audiogram is needed to confirm the degree of any hearing loss. How do you proceed with formulating a recommendation? How can you systematically approach such a decision?

EXAMPLE 2

A 70-year-old man with severe three-vessel coronary artery disease is being evaluated for coronary artery bypass grafting (CABG). An ultrasound demonstrates a 90% asymptomatic stenosis (a narrowing) of one of the carotid arteries leading to the brain. The decision faced by the team of physicians is whether to:

(a) perform coronary artery bypass surgery, without further diagnostic workup or treatment of the carotid artery stenosis;

(b) perform a carotid CT angiography to confirm the diagnosis and then a carotid endarterectomy (i.e., surgery to clear the obstruction in the carotid artery) prior to coronary artery bypass surgery;

(c) perform carotid CT angiography and if the diagnosis is confirmed then perform carotid endarterectomy during the same procedure as the bypass surgery.

Medical decisions must be made, and they are often made under conditions of uncertainty. Uncertainty about the current state of the patient may arise from erroneous observation or inaccurate recording of clinical findings or misinterpretation of the data by the clinician. For example, was the carotid artery stenosis really asymptomatic? Did the patient ever have a transient ischemic attack (temporary symptoms due to loss of blood flow to a region of the brain) that went unnoticed or that he interpreted as something else?

Uncertainty may also arise due to ambiguity of the data or variations in interpretation of the information. For example, if you repeated the ultrasound examination, would you get the same result? Uncertainty exists too about the correspondence between clinical information and the presence or absence of disease. The ultrasound is not perfect: how accurately does it indicate the presence or absence of a carotid artery stenosis? Some patients with a stenosis may be falsely classified as not having the disease, and some patients without a stenosis may be falsely classified as having the disease. Does our patient really have a carotid artery stenosis?

Finally, the effects of treatment are uncertain. In Example 1, there is essentially no diagnostic uncertainty, but there is uncertainty about the outcomes of treatment and about whether a trial of watchful waiting might allow the glue ear to clear up without medical or surgical intervention and without harm to the child. An important uncertainty, therefore, is the natural history of the disease. In Example 2, there would be uncertainty about the outcome of treatment, even if the diagnosis is certain and the treatment is well established. The rate of treatment failure may be known, but in whom it will fail is unpredictable at the time the treatment is initiated. For our 70-year-old patient we cannot predict whether performing a carotid endarterectomy will really protect him from a stroke during the CABG.

To deal with the uncertainties associated with the decision problem you need to find the best available evidence to support or refute your assumptions, and you need a framework for combining all of these uncertainties into a coherent choice. In a decision analysis process we first make the problem and its objectives explicit; then we list the alternative actions and how these alter subsequent events with their probabilities, values, and trade-offs; and finally we synthesize the balance of benefits and harms of each alternative. We shall refer to this as the PROACTIVE approach (problem – reframe – objectives – alternatives – consequences and chances – trade-offs – integrate – value – explore and evaluate) to health-care decision making. This has three major steps, each with three substeps. (The steps are a modification of the PrOacTive approach suggested by Hammond et al. in their book *Smart Choices* (4)). Though we present this as a linear process, you should be aware that often iteration through some steps will be required, and that sometimes the solution will be apparent before all steps are complete.

1.3 Step 1 – PROactive: the problem and objectives

You should begin by making sure you are addressing the right problem. This first requires that you make explicit what the possible consequences are that you are seeking to avoid or achieve. This may not be straightforward, as there are often different ways of viewing the problem and there may be competing objectives. Exploring these dimensions before analyzing the alternative actions is important to steer the analysis in the right direction. After the initial attempt at defining the *problem*, you should *reframe* the problem from other perspectives, and finally, identify the fundamental *objectives* that you are hoping to attain.

1.3.1 P: Define the problem

What are your principal concerns? A good way to clarify management problems is to begin by asking, 'What would happen if you took no immediate action?' This simple question seeks to uncover the outcomes that you might wish to avoid or achieve. Carefully answering this question should lead to a description of the possible sequences of events in the natural history of the condition. You may need to follow up by asking 'and what then?' several times. For example, a common cause of a very rapid heart beat is paroxysmal supraventricular tachycardia or PSVT (episodes of rapid heart beat initiated by the conducting system in the upper heart chambers). A patient with PSVT will typically experience a sudden onset of rapid heart beat (around 200 beats/min), which ceases suddenly after minutes to hours. It is usually accompanied by some anxiety, since patients worry that there is something very wrong with their heart, but it usually causes no

Table 1.1 Consequence table for the wait-and-see option for the problem of otitis media with effusion (glue ear)(6)

Consequences	Wait-and-see option
Hearing	Slow improvement over months to years
Behavior	Poor hearing may lead to disruptive behavior
Language development	Delayed articulation and comprehension (with possible long-term consequences)
Acute middle-ear infections	Recurrent episodes
Long-term complications	Possible conductive problems

other physical discomfort. If a patient presents after such an episode, you may analyze the problem by asking: 'What would happen if you took no immediate action?' A recent study in a cohort of nearly five million eligible patients demonstrated a statistically significant two-fold increase in the incidence of stroke in patients with PSVT compared to those without PSVT (5), demonstrating that the natural history potentially has dire consequences.

Other problems we will consider as illustrative examples in later chapters include management of needlestick injuries, smallpox vaccination, suspected pulmonary embolism, fatigue and iron deficiency anemia, imaging test for chest pain, testing for the BrCa1 gene for breast cancer, and atrial fibrillation. Each of these problems has a complex sequence of uncertain but potentially serious consequences. Visual aids that help describe the problem include decision trees, state-transition diagrams, influence diagrams, and survival plots. These descriptions are necessarily schematic: just as a map is useful to describe a territory, these visual aids help chart the possible course of events. They are helpful in describing and communicating the consequences and hence help navigate the decision-making process. The most straightforward tool to begin with is a *consequence table*, i.e., a tabulation of the principal concerns. Table 1.1 shows this for the management options for glue ear.

DEFINITION A *consequence table* tabulates the consequences of a choice and considers all relevant perspectives and important dimensions.

1.3.2 R: Reframe from multiple perspectives

Does the problem look different from different perspectives? You should understand how the problem you are dealing with appears to others. In the

clinical setting this requires that you broaden, at least temporarily, your focus from a disease framework to one that includes the concerns for the patient. In the context of public health this requires broadening your perspective to include the aggregate limits on resources, as well as the individual perspectives of the patient, the provider, the payer, and the public policy maker.

How does the problem of glue ear appear from different perspectives? You might consider different disciplinary perspectives. For example, biologically, glue ear is a problem of microbes, immune responses, and anatomical dysfunctions. From a psychological perspective, it is one of difficulties in language development. From a sociological perspective, it might be seen to be a problem of classroom behavior and family interactions. A public health practitioner may want to focus attention on adequate vaccination schemes to avoid infections. The child, the parents, the teacher, the primary care physician, the pediatrician, the public health practitioner, and the health-care insurance company will all view the problem differently and have overlapping objectives but with different emphases.

1.3.3 O: Focus on the objective

The main objective of health care is to avert or diminish the consequences of a disease. Sometimes this means prevention or cure; sometimes it may be slowing the disease's progress or preventing the disease's complications; sometimes it may be only the alleviation of symptoms or dysfunction. In our first example, only time will 'cure' the age-related anatomical problem with the Eustachian tube that leads to glue ear, but meanwhile you may alleviate the major problem – deafness – by removing fluid from the middle ear, or you may simply use a hearing aid.

If you framed and reframed the problem appropriately, the pivotal concerns and objectives should have become apparent. However, before proceeding to develop and evaluate options, you should check that you have a clear idea of the objectives. What elements are of most concern to the patient or population? What are the short-term and long-term objectives and concerns, and how do these vary between patients? Sometimes these objectives are straightforward. For example, the objective of immunization decisions is to reduce morbidity and mortality from infectious diseases. However, often there are multiple competing objectives. For example, in managing patients with advanced cancer there may be competing objectives of comfort, function, and length of life, and these may be different for patient and caregivers. If there are trade-offs between the objectives, it is obviously important to understand what the objectives are.

When listing the objectives, you should clearly distinguish between *means objectives* and *fundamental objectives*. A means objective is an intermediate goal but which is only a stepping stone to what we truly value. In our second example, the coronary artery bypass surgery is not a goal in itself, but a means of achieving the fundamental objectives of improved quality of life (less angina, i.e., chest pain) and avoidance of early mortality.

The nature of objectives may be clarified by repeatedly asking 'because?' or 'why?' In our first example, you might consider that insertion of a tympanostomy tube (grommet) will achieve the objective of resolving the glue ear, which may appear to be an objective. Why do you want the glue ear to resolve? Because that will lead to normal hearing. And why do you want normal hearing? Normal hearing will improve quality of life and it is important for proper language development. Why do you want proper language development? Because it improves quality of life. And why do we want to improve quality of life? That is something we intrinsically value, and hence it is a fundamental objective. Thus resolving the glue ear and obtaining normal hearing and proper language development are means objectives, whereas a good quality of life is a fundamental objective.

Understanding the fundamental objectives can help us generate options that achieve such objectives through different means. For example, focusing on quality of life instead of the fluid in the middle ear suggests that analgesics and a hearing aid may be good treatments to consider. Similarly, with the coronary artery bypass graft, you may need to step back and reconsider other options to manage the angina, such as stent placement or optimal medical therapy. Committing too early to a means objective rather than the fundamental objective can unnecessarily narrow our view of the possible options.

1.4 Step 2 – proACTive: the alternatives, consequences, and trade-offs

1.4.1 A: Consider all relevant alternatives

To be able to choose the best alternative in a particular circumstance, you need to know the range of reasonable alternatives. This list may be very long, so it is helpful to have a generic list. All alternatives may be placed in one of three categories: (i) a wait-and-see, watchful waiting, or a 'do-nothing' policy; (ii) initiate an intervention, e.g., treatment now; or (iii) obtain more information before deciding, such as ordering a diagnostic test or doing a population survey. These alternatives are illustrated in the decision tree of Figure 1.1.

The initial line is labeled with the population or problem you are considering (such as glue ear or coronary artery disease). The square represents a

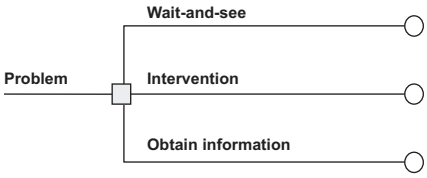

Figure 1.1 Generic decision tree for the initial decision node.

decision node at which just one of the several alternative actions, represented by the subsequent lines, must be chosen. At the decision node, the decision maker is in control. From each alternative action, there will usually be a subsequent chance node (the circles), with branches representing the possible outcomes of each option. The probabilities of events and the ultimate outcomes will depend on the alternative chosen. Let us look in more detail at each of the three generic alternative decisions.

DEFINITION A *decision tree* is a visual representation of all the possible options and the consequences that may follow each option.

1.4.1.1 Wait-and-see, watchful waiting, or do-nothing policy

A wait-and-see, watchful waiting, or do-nothing policy may take several forms. You may decide to do nothing about the condition. For example, this might be a reasonable choice for benign skin lesions or other variants of 'normal.' However, usually you will have a contingent policy that requires action depending on the disease course over time. The contingencies may be classified as either *monitoring*, where a regular check is made, or *triggering*, where you wait for a change in the type or severity of symptoms.

With monitoring, a check is made at fixed times to see whether the condition has improved, remained the same, or become worse. Action is then based on this progression. For example, you may decide not to treat patients with mild hypertension until their blood pressure increases or they develop other risk factors; the criterion for action is the condition becoming worse. For the glue ear case, you may decide that action is required if no improvement is seen at two months; the criterion is either no change in the condition or a worsening. If a condition is unchanged, why should its persistence indicate a need for action? Imagine that there are two types of the condition: those that spontaneously resolve and those that never resolve. Waiting will allow us to differentiate these. Effectively this is a test-of-time. In reality, the groups will not be so distinct, and the test-of-time will be imperfect. So there will be a trade-off: delay may reduce the benefits for the persistent case but

avoid the harm of unnecessary treatment for those who would resolve spontaneously.

With triggering, the patient is advised to return if particular events occur. In family practice this method is known as safety netting – a patient is instructed in the criteria required to catch a potentially ominous change. Clearly, wait-and-see is a strategy rather than a single action. Thus a *strategy* is in fact a sequence of choices contingent on the observed events at chance nodes. In some cases it may be useful to consider several different wait-and-see strategies.

1.4.1.2 Intervention

The next step is to list the active intervention alternatives, refraining from any evaluation of their merit at this point so that the full range of options can be considered. In the glue ear example, intervention would be treatment which may be aimed at cure, at arresting the progress of the disease, at preventing complications, or at alleviating the symptoms. As described earlier, glue ear may be managed by attempting to resolve the effusion (cure), or by prescribing analgesics and use of a hearing aid, which would alleviate the principal symptoms of pain and hearing loss and the consequences.

Where do you get the list of alternatives? Websites, mobile applications, discussions with colleagues and experts, textbooks, and literature searches all contribute. An important component is a search of controlled trials, since these are often the source of the best-quality evidence on the benefits and risks of interventions. The Cochrane Library is a good place to start: it contains systematic reviews of randomized controlled trials (RCTs) and references to RCTs. A search of the Cochrane Library for 'otitis media with effusion' (performed in Sept 2013) provided 12 systematic reviews and 532 RCTs that include: (i) antibiotics, such as ceftibuten, cefixime, amoxicillin, and cotrimoxazole; (ii) oral corticosteroids, such as betamethasone, prednisolone, and prednisone; (iii) intranasal corticosteroids such as beclomethasone; (iv) nonsteroidal anti-inflammatory drugs, such as naproxen and tranilast; (v) tympanostomy tubes (ventilation tubes/grommets) with two major different types; (vi) adenoidectomy; (vii) mucolytics such as carboxymethylcysteine and bromhexine; (viii) autoinflation (mechanical maneuvers which force air up the Eustachian tube); (ix) decongestants and antihistamines; and (x) hearing aids. Some of these options, such as antihistamines, are clearly ineffective. Others, such as mucolytics, autoinflation, and nonsteroidal anti-inflammatory drugs, are of doubtful or uncertain value. The remaining treatments show a range of effectiveness and harms, which need to be compared.

Figure 1.2 shows the start of a decision tree for our second example. In this example, the do-nothing option is to refrain from treating the carotid artery