Fairness is at issue

Ruth Chadwick

The last paragraph of Bochner et al's paper asks whether the model and its application are fair. The answer is said to depend on whether rationing itself is fair. And yet arguably the very concept of rationing (as in postwar rationing, for example) involves sharing resources equitably, giving each person their portion. How to achieve an equitable distribution is the issue. In the current debates about allocation of health care resources, however, the term "rationing" has acquired a bad image, whereas "priority setting" has for some reason been thought preferable, although setting priorities precisely does mean giving preference to some areas and thus some people, with the implication that others will go without.

"Interpersonal comparisons... affect most if not all criteria of allocation."

The model outlined is one that gives priority to some drugs over others—some will not fall within the budget at all. Is this fair? The question turns on what criteria of allocation are used. A procedural requirement of fairness is that like cases are treated alike: the authors admit the desirability of consistency in decision making. There is a question, however, as to whether consistency in treatment of drugs is what is desirable, rather than consistency in treatment of people. Although it is claimed that cost was not allowed to override other considerations to the extent that certain patient groups would be denied potentially life saving though expensive treatment, the system proposed will surely have this effect at times: some people will not receive what they need. Concentration on choosing between drugs distances the decision makers from this fact.

There are strong, though not universally accepted, arguments for the view that distributing resources according to need is what is fair. In Bochner et al's paper, however, the first guiding principle at an operational level is based on the "need to obtain the greatest benefit for the most patients for each dollar spent." In what sense this is a "need" is not made clear. Further, as in the case of the utilitarian principle of maximising the greatest happiness for the greatest number, this looks like one principle but in fact contains two parts: "greatest benefit" and "most patients." The fact that these can conflict is disguised by the example of a choice between 55 patients at $16,000 and 4000 at $5000. What if the choice is between 55 and 4000 for the same cost, where the 55 gain a much greater benefit than the 4000? This presents starkly the clash between "greatest benefit" to a few and the smaller benefit to "most patients."

This issue gives rise to the problem, recognised by the authors, of the difficult issues surrounding interpersonal comparisons, which of course affect most if not all criteria of allocation. Measurement by QALY is rejected partly on the ground that it is "likely to be as subjective and potentially inaccurate as the method currently used." This hardly seems a strong argument for preferring the latter. Intuition is appealed to as a rationale for according a score of 30 to a treatment that results in cure or prevention of an undesirable outcome while one that prolongs life receives 15. There are several problems here. First, what is meant by "cure" and "undesirable outcome"? How is cure distinguished from prolonged life? Even if cure is preferable, is it twice as good?

Some aspects of the description of the model give cause for concern. For example, it is said that there is a tendency to underestimate the number of patients "worthy" of a new treatment. What does "worthy" mean in this context? Finally, the list of interested parties to consider includes patients but does not include society, or the public, who surely have an interest in how health care resources are allocated and arguably should have a voice in decisions on criteria of allocation.

Authors' response

We have the impression that our commentators are theorists and have not had the responsibility of managing a capped drug budget in a climate which rightly demands the introduction of new—and often very expensive—drugs. Perhaps they failed to appreciate that the model is only one of several strategies to improve drug use; it was developed to respond to our hospital's restrictions on the drug budget, with the consequent need to impose some rationing to avert an even greater crisis than might have otherwise occurred. This was not some theoretical game, the playing of which involved the luxury of prolonged debate, philosophical meanderings, and testing several economic models.

Cam Donaldson has accused us of creating a formula which lacked clear thought. The elements in our equation were based on the information in table II which we believe contains key concepts to allow a judgment to be made about setting priorities for drugs—and for the patients needing them. Some elements of this information are more objective and therefore more easily quantified than other inevitably more subjective elements, but the best available contemporary evidence is used to decide on the allocation of scores, especially in the numerator. We must emphasise that the scores have no inherent value apart from facilitating ranking of requests. Thus, rather than hiding subjectivity, the equation imposes a rigour in the decision making process. At least our clinicians now understand the reasoning behind the final decisions, and actively participate in them. Does Cam Donaldson seriously suggest that total cost should not be counted at all? This statement ignores the reality of the world in which some of us have to function. We agree that priority setting is about making judgments. This model has facilitated the process in our hospital.

Ruth Chadwick echoes many of the concerns we had during the model's gestation. Although the model concentrates on drugs, it equally takes into account the treatment of people and treatment outcomes (see table II, especially items 2-9). She suggests that the model will deny potentially lifesaving drugs on cost grounds. The introduction of the model has had the opposite effect since this was already happening in our hospital. We accept that the guiding principle behind the model contains two parts. However, the model would accommodate the example cited by Ruth Chadwick, since it is likely that the score generated from the greater benefit experienced by the smaller number of patients would offset the score in the reverse situation. We agree that the word "worthy" carries judgmental overtones; in this context it means those patients who qualify according to the criteria in table II.

We agree with much of Petrie's commentary. The drug committee sees itself very much as an advocate for the patient and prescriber. The introduction of the
Why we need a clinical trial for vitamin K

J M Slattery

Vitamin K is given to many babies born in the United Kingdom, but we still do not know if it has substantial hazards. Because the population exposed to vitamin K is very large even quite small hazards would involve many adverse events. It is therefore important to be able to put reasonably close bounds on the potential damage that vitamin K prophylaxis could cause. Past research has not allowed us to do this but a large randomised controlled clinical trial of vitamin K against no vitamin K, enrolling only infants at low risk of haemorrhagic disease, would do so. There is no question that vitamin K is a useful treatment in babies at highest risk of haemorrhagic disease: the question is whether the trend towards use of vitamin K in lower risk babies should be encouraged.

Vitamin K has been widely used for over 30 years as a prophylaxis against haemorrhagic disease of the newborn, but the first case-control study of its long term safety (in terms of the risk of cancer) was not published until August 1992. This suggested that vitamin K given intramuscularly to neonates might double the risk of childhood cancer.1 More recent Swedish and American studies have not shown similar effects.2 Of course, all effective treatments carry some risk, and to assess whether widespread use of a particular treatment is warranted we must know both the risk of not treating and the risk of treating. Furthermore, if we can identify groups of neonates with more or less to gain from treatment we must know the risks within each such group. Randomised clinical trials are by far the most reliable method of assessing the balance of risks and benefits of treatments but a major difficulty is finding a form of clinical trial that is informative, practicable, and acceptable to parents and medical staff. It is most unlikely that a trial in infants at high risk of haemorrhage would ever be considered ethical unless convincing evidence of adverse effects was discovered. In this paper I argue that, among infants at low risk of haemorrhagic disease, we are never likely to discover the true balance of risks and benefits but that a well designed large clinical trial could provide acceptable bounds to our present state of ignorance and provide better information to allow parents to make an informed choice of treatments.

Clinical research

The largest clinical studies of vitamin K were carried out nearly 50 years ago. A review by Dam et al reports the work of Dyggve who compared the clinical outcome for 11 000 babies whose mothers were given oral vitamin K within 48 hours of the birth with 22 000 controls.4 Lehmann compared 13 250 babies given oral vitamin K with 17 740 historical controls.5 Neither study was a randomised controlled trial, but both had large untreated groups that provided estimates of incidence. Lehmann’s primary clinical outcome was death from haemorrhage while Dyggve reported all haemorrhages. This difference complicates comparison of the studies, but where comparable measurements were made the results were quite different. For instance, in combined treatment and control groups Dyggve reported 411/33247 (1.23%) deaths from intracranial haemorrhage, and Lehmann reported 54/30950 (0.17%). The control group frequencies from these studies were 4.2 haemorrhagic deaths per 1000 (Lehmann) and 36.3 haemorrhages per 1000 (Dyggve). The sevenfold difference in mortality from intracranial haemorrhage leads us to view these figures with caution, but there were clear differences between the studies that might explain this; for instance, Lehmann selected infants weighing over 2500 g while Dyggve could not have applied such a criterion since treatment was antenatal. Both studies found a statistically significant and clinically important reduction in haemorrhage in treated babies, but neither reported any long term follow up necessary to pick up a side effect such as cancer. Lehmann’s study is more relevant to current clinical practice since vitamin K was given directly to the infant.

There have been surprisingly few recent studies, and the Oxford Database of Perinatal Trials lists only two which measured a clinical outcome.6 Vietti et al restricted their study to bleeding complications after circumcision within days of birth, and hence it sheds little light on the desirability of prophylaxis for infants with low risk of haemorrhage.6 Sutherland et al randomised 3338 infants into three groups: placebo, 0·1 mg menadione, or 5 mg menadione given intramuscularly.7 The babies were of normal birth weight (more than 2260 g), and so this study is comparable with that of Lehmann. Any bleeding within the first four or five days of life was recorded: 86/1143 (7·9%) with placebo, 60/1132 (5·3%) with 0·1 mg menadione and 61/1063 (5·7%) with 5 mg menadione. The two doses of vitamin K were equally effective even though one was 50 times smaller than the other. Of these bleeds, 68% were associated with circumcision and many were fairly minor. There were eight serious bleeds in the placebo group (0·7%) and none in either treated group. Four of these eight were associated with circumcision so that an estimate of serious bleeding in normal birthweight, uncircumcised infants is 3·5 per 1000.

I have concentrated on haemorrhages with a lasting impact on the health of the child because I am assuming that the adverse effect we are most likely to be interested in is cancer. Hence, the benefit we should consider is avoidance of a similarly serious disease.

Late haemorrhagic disease

Haemorrhages more than a week after birth were not well documented before the introduction of prophyl-