

Commentary on papers by Raanan Gillon and Paul Hansen on High-cost pharmaceuticals review

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Introduction

This commentary is written by a non-medical person who will approach the topic from a consumer perspective. However, it is my own personal view.

1. Background to resource allocation

Systematic explicit approaches to resource allocation have been attempted before in New Zealand, most notably by the now disbanded Core Services Committee. This involved a number of public consultations including ‘town hall meetings’ which canvassed the views of the public. The Health Funding Authority also began a process of ‘reprioritisation’ before it too was disbanded.

In general, these processes have been fiercely contested and no consensus emerged. It is clear that there is no simple answer to the issue of allocation of constrained resources, and no accepted methodology which is able to reflect the scope and complexities of the range of approaches and values that are brought to bear on this issue.

The author of this commentary similarly does not come up with a path out of the maze. Rather, the focus of this brief paper will be on highlighting issues that arise from the reports by Gillon and Hansen, from a consumer perspective.

2. High-cost medicines, all medicines and the wider health sector

I would like to raise the distortion that occurs through looking at high-cost pharmaceuticals in isolation from all pharmaceuticals, and, taking an even broader approach, the distortion that occurs when medicines are subject to rationing differentially from other areas of the health care sector.

2.1 High-cost medicines and other pharmaceuticals

Looking at the wider context of pharmaceutical use, there is a degree of over-use of pharmaceuticals in particular areas. I will give an example of one area with which I am familiar. Through the 1990s, many thousands of mid-life New Zealand women began to use hormone replacement therapy for prevention and ‘wellbeing’. This was the result of aggressive marketing by the pharmaceutical industry, as well as promotion by medical opinion leaders and uncritical dispensing by prescribers. A study in 1997 showed that 20 percent of mid-life New Zealand women were using HRT, an increase from 12 percent in 1991. There was also an increase in long-term use for prophylaxis (North and Sharples, 2001). In 2001, after several decades of use, HRT was definitively shown to cause harm. I am not aware that anyone has quantified the cost to the Vote: Health of the over-use of HRT that occurred for the decade or so

before the publication of the Women's Health Initiative results in 2001, but it would be an interesting exercise.

There are other examples, especially in areas where a 'mass treatment' approach has been taken. The mass treatment of hypertension and hypercholesterolaemia in the 1980s are examples. (Jackson and Kawachi, 1992, and Kawachi, 1992).

Over-use can also occur for historic reasons. Pharmac has tried to curb widespread inappropriate use of antibiotics and, historically, there has been an overuse of some types of pharmaceuticals amongst the elderly, in particular, anti-depressants and sleeping tablets.

In summary, there is still room for improvement in the prescribing of drugs other than high-cost drugs. Such an improvement is largely beyond the role of Pharmac, as it involves such things as regulation of the activities of pharmaceutical companies, funding for clinical research, medical education, consumer information, and the current structure of primary health care.

More rational and judicious prescribing behaviour would alleviate pressure on the whole pharmaceutical budget, such that the use of high-cost pharmaceuticals might not be so problematic.

2.2 High-cost medicines and the wider health sector

There is no logical reason why high-cost medicines should be subject to a set of rationing criteria which do not equally apply to other agencies and health care services, such as treatment services, and the activities of the Accident Compensation Corporation.

The 'booking system' is a form of rationing in the treatment sector, but it only applies to particular treatments, whereas others are not rationed, or at least, not explicitly.

Some very costly treatments, such as neonatal intensive care, are 'over-used', having evolved into almost a competition to salvage the lowest weight babies.

There is no logic to spending six figure sums on severely compromised neonates or people injured in traffic crashes, but denying costly pharmaceutical treatment to others, who may well benefit more, simply because they fall within a different funding silo.

If rationing is to be practised, it should occur evenly across the health sector. I am not convinced that it need occur at all, if more attention was given to practising evidence-based medicine. I am not aware of any work that has occurred to evaluate whether an evidence-based approach would result in resources going further, but I'd like to believe that they did.

If prescribing was more constrained and rational (evidence-based), there could well be savings that could be applied to widening access to high-cost pharmaceuticals that do benefit people, who are often facing serious health problems.

3. Should high-cost medicines be available?

I am in general agreement with the proposition that high-cost medicines should be more available. Even the term 'high-cost pharmaceuticals' is contestable. The level at which Pharmac considers a medicine 'high-cost', differs from some other countries. For example, NICE seems to regard high-cost medicines as those with a cost-effectiveness in excess of 20,000 to 30,000 pounds per QALY (NICE 2005). In other words, there seems to be little agreement as to what constitutes 'high-cost', so that treating high-cost medicines differently from other pharmaceuticals is a proposition that is in itself open to challenge and debate.

There is a strong case to be made for making high-cost medicines more available. People needing high-cost medicines often face conditions or diseases that are rare, life-threatening or have a major impact on their lives.

It would appear at first glance that in this case the decision criteria should be weighted towards the degree of benefit to be gained. If the degree of benefit is significant, then that would justify the greater cost. It would even be possible to argue that a great benefit should be required of high-cost medicines.

However, it is not that simple. Indeed, it may be that for at least some high-cost medicines, weaker evidence, or less benefit, might be acceptable given the situation of many of the people seeking high-cost medicines.

Some of these high-cost medicines are, at least initially, supported by less than robust evidence. While I would ordinarily argue in favour of having good quality evidence for safety and effectiveness before scheduling a medicine, I think an argument can be made for relaxing the standards when people are facing dire consequences. When people are in extremis, such as facing death, they may be prepared to accept a greater degree of risk, and I would probably argue that society should support them in these actions. (This should be distinguished from supporting interventions that are futile).

High-cost medicines may inherently have less robust supporting evidence. They are usually developed to treat conditions that are relatively rare, and/or those that have shown themselves to be intractable to other forms of intervention. The situation of people who are seeking such medicines is often urgent. Consequently, there is an argument that they do not have the luxury of waiting for the accumulation of the good quality long-term studies that we would normally expect to support the approval of a subsidy.

I am not coming to any conclusion here, rather I am putting forward arguments as to why one might consider a different approach to high-cost pharmaceuticals.

4. What outcomes should be sought?

I am in broad agreement with the proposition by Gillon 'that equals be treated equally and unequals be treated unequally'. Both Hansen and Gillon cite Rawls' theories which would probably best express my own viewpoint.

Pharmac decision criteria do not explicitly require it to reduce inequalities, but the agency is required to include the Government's priorities for health funding. A principle in the *New Zealand Health Strategy* is 'an improvement in health status of those currently disadvantaged' and to this end the Strategy seeks accessible and appropriate services for people from lower socioeconomic groups, Maori and Pacific people (Minister of Health 2000).

Ministry of Health research shows that there are significant ethnic group differences in access to medicines. In a 12-month period, European/Pakeha adults were more likely to be prescribed 10 or more items than adults in the Maori Pacific and other ethnic categories. This is surprising when considering that Maori and Pacific people have poorer health status than Pakeha, and Maori are more likely than Pakeha to have six or more visits to a GP in a year (Ministry of Health 1999).

Therefore a case might be made for greater access to high-cost pharmaceuticals for these groups who have received less at the primary care level, and whose health status is generally lower. . The argument for distributive justice could support greater accessibility to high-cost pharmaceuticals for those who have missed out on first-line treatment, or who are the victims of structural inequality. Because differentials based on ethnicity are generally unpalatable to governments and to the public, this could, in a circuitous way, bolster the case for a general widening of access to high-cost pharmaceuticals.

Cost Utility Analysis inherently discriminates against those with chronic illness, people with some disabilities, old people and others who will never get 'well'. The quality of life of some of these groups may already be compromised and these same people are often subject to other forms of discrimination and marginalisation in their daily lives. In the case of the elderly, they are means tested before receiving care which does not occur with any other section of the population.

In the case of the elderly, or others, I am not arguing for heroic and futile treatment, but for ready access to pharmaceuticals which will improve their quality of life, even if they do not prolong it or even if they cannot cure underlying conditions.

Indeed, any system of allocating health resources should not place undue emphasis on prolongation of life, at the expense of quality of life in the present. The QALY system inherently does this.

Even the concepts of improving health status or addressing 'need' are problematic for already compromised groups such as those I have discussed. For example, imagine a drug was developed that would assist continence in the elderly. Would a person with dementia, in a wheel chair, have improved health status if treated? Would their 'need' be greater than a person who could get back to work if given a particular drug? Probably not, but there would be improvements in the degree of comfort of the person, there would be less likelihood of rashes and skin irritation, and it would be more feasible to take them on trips outside the institution thus enhancing their quality of life.

The person's autonomy and dignity would be respected, and as a society we would be showing that all human life is valuable, especially our most dependent and vulnerable members of society.

As Gillon argues, the priority of pharmaceutical policy should not always be to save life ahead of other priorities for improving the quality of people's lives.

There will be a range of views about this among the public, but very often the public does not support saving life at all costs. I was struck the other night by a mother interviewed on TV (in the case of her child damaged during birth) who said she had hoped her child would not live, and even though she now loved him greatly, she still wished he had not lived because of the low quality of his life and his very bleak future.

I think we often do not give the public due credit for their understanding of the complexities of these issues, and their acceptance that sometimes saving lives condemns those saved to a life of suffering.

5. The rule of rescue

There are real dilemmas around the urge of society to save people under imminent threat. While, as Gillon says, it is to a degree 'instinctive', it is in our society highly manipulated and can result in actions which exacerbate inequalities.

The rule of rescue is exploited by the media which in recent years loves a 'human interest' story especially one which involves bashing unfeeling bureaucracy. Examination of the evidence for effectiveness comes second to the human drama.

More recently, the internet is being used to build support for particular sad cases. While this can be seen as 'democratic', as it is accessible to many people and it is unmediated by any authority, it can also be a forum for the exchange of uninformed, inaccurate information, and it appeals to other aspects of the human character than reason and logic. This is not necessarily a bad thing, as human beings should give weight to values such as compassion, community, charitableness, and kindness to strangers. However, it can build into public campaigns that benefit particular individuals, regardless of the actual merits of the case, and leapfrog over other people with less compelling and dramatic cases, or with fewer resources to draw their situation to public attention.

There are strong moral and social status elements in most appeals to the rule of rescue. The disease is usually seen as particularly tragic and random, and the sufferer has usually done nothing to bring it down on themselves. For example, it would be hard to build a campaign around a still-smoking sufferer from lung cancer.

The sufferer is often educated and middle class with social networks that are able to bring many professional and personal resources to a campaign.

For these reasons, responding to these situations has the potential to exacerbate existing inequalities in health status and access to services.

On the other hand, sometimes, this is all people have left to do, and they are facing life and death issues. A rigid application of rules runs the risk of demeaning society as brutal and uncaring. I do not think anyone gained from the refusal to give dialysis to Rau Williams, which was traumatic and depressing.

In this case, politicians supported the stance of the bureaucracy. On other occasions, they overturn decisions of the bureaucracy. Some see this as a bad thing, and sometimes it is. But it is the role of politicians to step in and intervene when bureaucracies are acting in rigid and inhumane ways. Politicians are accessible to the public, and accountable to them. After all, all our bureaucracies are the creations of politicians, so they do have a duty to listen to the public, assess the situation and intervene on behalf of the public when they think the bureaucracy has got it wrong. The bureaucracies are the servants of the people, not their master.

The way in which society cares for its most vulnerable is an important measure of how humane and mature we are as a society. This is recognised in many aspects of our social, legal and political structures and in our daily interactions with each other as human beings. Adults in general watch out for children, even where they are not known or related. In general, we support treating elderly people with respect and care, so they are comfortable in their final years, although we do not always do this very well. There is general consensus that we provide support and resources for people with disabilities, who are sick or who have suffered misfortunes such that they cannot be totally self-supporting.

Although some of these community values have been eroded in recent years, they are important, and government policies and practices should bolster rather than erode them.

6. So how do we decide about high-cost pharmaceuticals?

Hansen makes the point that Pharmac's existing methodology is focused on outcomes, and that an economics-based methodology is utilised to inform decision. The Cost-Utility Analysis is concrete and well-developed. This is but one aspect of the decision-making, and other aspects can be brought to bear on final decisions by Pharmac.

However, because the other factors that can be considered are vague and not codified, I suspect that Cost-Utility Analysis is fairly determinative. (I concede that I have not seen any research to back up this assertion.)

The process by which decisions are finally taken is not transparent, and they are not (that I am aware of) well documented and therefore open to scrutiny.

Hansen's proposed mathematical approach to making decisions, and the hypothetical points system for deciding which pharmaceuticals to fund (Figure 3, p 23) do not sit comfortably with me. Allocating points on the basis of age is repugnant and I would think illegal within the New Zealand human rights framework.

While a quantitative system is superficially attractive, there is little evidence it will lead to good decisions. It may even act as a refuge, allowing us to avoid taking

responsibility for the decisions that are made. We must never lose sight of the fact that real people with families and friends are affected by such decisions. If we were to have a tick-box system, can I suggest that the final line is:

‘Would you want your child/father to have access to this drug if he needed it?’

I would argue that because such decisions critically affect human lives, it is better that they are made through a more deliberative process, that allows other aspects to be considered alongside cost, efficiency and cost-effectiveness.

I am arguing that making such decisions should be values-based, rather than made according to a formulae. While assessment of costs is important, and should be part of the decision-making, it should only be one part and there should be an explicit process for ensuring that other values play a significant part.

It is also important to be more explicit and open about the process. Decisions should preferably be made in public, or at least made public, and the basis of the decisions needs to be well explained.

I am arguing that the process by which these decisions are taken is just as important as the outcome. I would hope that if people understand and respect the process, they would understand and support decisions that are taken.

I am making an argument for a much more people-based system. One where the decision-makers know just who they are affecting, and where the decision makers are known and accountable for their decisions.

Gillon recommends the creation of an ‘allocation committee’ although he sees it as advisory to Pharmac rather than making decisions. He suggests looking at models such as ethics committees, and the NICE Citizens’ Council.

There are merits in the Citizens’ Council model. It is an attempt to incorporate the values of the public. Efforts are made to ensure that the council is perceived as having independent standing and is not a tool of the allocation entity. The selection of the councillors is undertaken at arms’ length from NICE, meetings are held in public, and councillors help choose witnesses and question them.

I personally have some issues around the make-up of such a council, and members’ accountability for the effects of their decisions.

A Citizens’ Council is constructed of notional demographically representative citizens, so that the council mirrors the make-up of society by age, ethnicity, gender etc. However, a single person cannot represent the totality of the viewpoint of all those in the same demographic group, so that it is questionable how ‘representative’ such a committee can be.

I would prefer, rather than the unaligned ‘man or woman off the street’ approach, that people are nominated by respected community organisations, so that they have access to a broader range of views than their own, and they have experience of a constituency. While fears have been expressed that representatives of community

groups will only advance the agenda of their groups, this is not so in practice. Using people who have a constituency has been shown to result in better decision-making than the 'lay person' approach, and there is evidence that the public recognises representatives of civil society as representing their interests (Coney 2004).

I am not sure the exact Citizens' Council model would be acceptable in the New Zealand context. The UK puts great store in the NHS on the notion of citizenship. Such a concept is somewhat problematic in New Zealand because of the existence of the Treaty of Waitangi.

Another alternative would be to elect people so that they are directly accountable back to the public they represent.

There is value in having some continuity of individuals on any allocation entity. There is merit in looking at the composition of ethics committees as one form an allocation group could take. Without being categorical about this, I think people with ethical, legal, human rights, and other backgrounds should be included in any allocation process. Consumer representation should also be included.

Some means of accountability for the panels should be developed. This could include a publicly known process for appointments, opportunities for people to express an interest in being included, as well as nomination by various bodies, such as health professional colleges. The general features of decisions should be publicly reported, and the panel could publish an annual report, summarising the year's work. It would also be important that such a council deliberated in public, to reinforce transparency and accountability.

There could well be few takers for such positions!

Gillon also recommends making explicit the ethical framework in which Pharmac works and I support the development of such a framework. I would see Pharmac as needing to consult widely in the development of a framework.

In doing this, Pharmac could well look at the recent *Social Values Judgements: Principles for the development of NICE guidance* (NICE December 2005). These provide guidance on how to incorporate social values into allocative decision-making. They were developed from a review of published literature, reports of the Citizens' Council and a telephone survey of the public about the role of NICE and attitudes to priority setting. They provide a basic set of 'bottom-lines' for decision-making to safeguard against decision-making being discriminatory or judgemental. For example, Principle 7 states that decision-makers must not distinguish between individuals on the basis of gender or sexual orientation. (New Zealand might come up with a different set of principles than these, so I am not arguing that the NICE principles are simply transferable).

I think the development of a similar framework as a first step would assist Pharmac in approaching the current topic, but it is essential to do this with external stakeholders and the public, not as an internal project.

Gillon also recommends an appeal mechanism and I would support this.

The recent NICE Social Values Framework arrives at characteristics for strategies for setting priorities that I found very attractive, and the section of the report dealing with this is worth copying here.

‘2.2 Strategies for setting priorities

There is a groundswell of opinion among bioethicists and political philosophers that, if there is to be confidence in the legitimacy of decisions, the procedures adopted should have all four of the following characteristics:

- Publicity
- Relevance
- Revision and appeals
- Regulation

2.2.1 Publicity

Decisions about limits on the allocation of resources should be made public. This includes not only the decisions themselves, but also the grounds for making them. It does not, however, require that all criteria for decision-making should be established in advance: rather, there should be room for the development of “case-law”.

2.2.2 Relevance

“Relevance” means that the grounds for decisions are ones that fair-minded people would agree are relevant to meeting healthcare needs, especially where there are constraints on resources. In particular, ‘relevance’ focuses on the importance of deliberation about the limits of the common good and acknowledges that such “deliberative democracy” should involve both the decision-makers themselves and those whom the decisions affect.

2.2.3 Revision and appeals

There must be opportunities for challenging decisions and mechanisms for resolving disputes. There should be system in place for revising decisions when new, or additional, evidence becomes available or new arguments are put forward.’

2.2.4. Regulation

There should be either voluntary or public regulation of the process of decision-making to ensure that it has all three of the above characteristics (publicity, relevance and opportunities for revisions and appeals.’

2.2.5 Accountability for reasonableness in decision-making

Ensuring that procedures have all four of these characteristics makes decision-makers “accountable for their reasonableness”. Critics claim that majority preferences – however well-informed and fair – will sometimes lead to unjust outcomes, that deliberative democracy in action will “most certainly” conflict with the principles of justice, and that “deep suspicion is warranted about procedural strategies for setting priorities”. Such criticisms have some merit: yet no reasonable theoretical or practical alternatives have been proposed to resolve the conflicting theories of distributive justice.’ (NICE 2005)

As a step towards a process that has public acceptance, Pharmac could develop a discussion paper with options for strategies and structures for make recommendations about high-costs mechanisms. This could put forward a number of principles and models, drawing from overseas, but shaped for New Zealand’s unique circumstances.

A public dialogue would engage the public in this issue as well as raise public knowledge of its complexities. Independent research, such as that commissioned by NICE, could also assist the process. This would be first step towards developing a process for making high-cost allocation decisions that is known and accepted by the public as a fair way of approaching this very difficult subject.

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