



EMBARGOED until noon Tuesday 7 June 2011

20 Questions about funding medicines for rare “orphan” diseases

1. Should patients with life-threatening diseases that have no treatments currently available to them, be denied access to new therapies because of a narrow focus on the cost-effectiveness of the medicine compared to other medicines?
2. Should patients with very rare “orphan” diseases miss out on effective treatments because tiny numbers (one for one disease, four for another) make the unit cost of treatments much higher than typical medicine costs for much larger populations with common diseases?
3. Should the cost per patient, which is high, be considered in isolation from the total cost to treat the very small number of patients with these diseases, which would NOT be a large cost?
4. Should patients relying on the exceptional circumstances process for consideration of their medicine funding application, have to face the reality that budget management and cost effectiveness are carefully evaluated by Pharmac, but the moral dimensions of fairness and community values are ignored?
5. Is it acceptable that a decision is made to deny funding when the decision papers note the improvement then stabilisation that is likely to occur for a degenerative disease, and notes there is no other treatment for the life-threatening disease?
6. Some diseases are not treatable. That is a reality we may all face. When some become treatable but funding is denied the effect is to deliver a death sentence through denial of treatment. Is that acceptable?
7. One of our military servicemen has come home from Afghanistan to find our health system will not treat his wife’s life-threatening condition. Is this acceptable in our society?
8. Should adults with a life-threatening degenerative muscle disease have to read reports of improvements in health and quality of life of treated patients elsewhere in the world, some getting out of their chairs and taking up skiing again, and know that our country will not treat them?
9. Why does our medicine funding system not provide for some balance against the inherent disadvantage of rarity of a disease? We provide balancing consideration for many other things including social, economic and cultural disadvantage. Is “too small, too bad” an acceptable response?
10. Pharmac staff and Board are dominated by accountants, managers and health economists. Is it acceptable for these types to drive policy and implementation regarding medicines when they have no moral philosophers or ethicists among the decision makers or advisors?
11. Is it acceptable for Pharmac to deny a medicine for a life-threatening disease solely on the basis of the cost per “quality adjusted life year” and exclude social issues and equity?

12. Is it acceptable for Pharmac to ignore the specific provisions in the medicines strategy that state fairness and community values are to be incorporated into decision making at all levels in the medicine system?
13. Why should patients accept decisions to decline these therapies when they regularly read of decisions to fund many millions of dollars for relaying grass at AAMI stadium, contributions to boat races, costs of the BMW fleet, and many other discretionary funding decisions by government?
14. Is it acceptable for papers on which the decision to decline was based, to have excluded advice to the Pharmac Board about the discounting offer made by the major supplier of these medicines?
15. Are the costs of these medicines really unaffordable when the discounted cost per patient is likely to be less than the costs per year of a maximum security prisoner?
16. Should it be acceptable for medicines to be refused because of price, when government funded research institutions are working hard to discover treatments and are expected to capture the intellectual property so we can reap the income from new products in high value medicine markets?
17. What decision would you make about funding these medicines if the patient was your child, your parent, your husband or wife?
18. Many millions of medicine funding are spent on those who need treatment because of risk-taking behaviours, or because they have neglected to maintain a healthy lifestyle. We do not discriminate against them in the medicine funding system, but those with inherited genetic conditions that cannot be avoided, are effectively discriminated against because the rarity of their disease leaves them out of consideration. Is that acceptable?
19. Many interest groups have repeatedly advised government that Pharmac has a narrow focus that makes it inappropriate for them to be charged with setting the high-level policy on access to specialised medicines as well as managing the day-to-day purchasing processes. Why are these messages not being listened to?
20. **Will the government intervene to put in place a fairer system for access to medicines for orphan diseases? Australia has one. Why can't we?**

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