

28 July 2011

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Dear John

**Named Patient Pharmaceutical Assessment (Exceptional Circumstances)**

I refer to your e-mail dated 8 July 2011. Pharmac has released its new Exceptional Circumstances Policy, and you have asked me to review the new policy and your initial thoughts, which you e-mailed to me.

I think everything you have said in your e-mail is correct, and they are all informative points that are worth recording. In particular, I agree with the comments you have made about the effect (or not) of the submissions made to Pharmac by NZORD and others.

There are only a few other things which I would also say by way of emphasis from my perspective. Following a similar format to your e-mail, my comments are as follows.

1. The basic framework between the consultation version of the Exceptional Circumstances Policy and the version which has now been promulgated remains the same. There are three “prerequisite” or “eligibility” pathways which provide an initial filter, and then there is an assessment by Pharmac using its standard assessment criteria. The only substantive changes made by Pharmac relate to the specifications of the first two pathways (the Unusual Clinical Circumstances and Urgent Assessment pathways), and to some of the descriptions around the edges of the Exceptional Circumstances Policy.
2. The explanation of how the Exceptional Circumstances Policy sits alongside the Pharmaceutical Schedule is clearer in the new policy than previously. In particular, I think the explanation that the exceptional circumstances are an exception to the circumstances (and pharmaceuticals) covered by the Pharmaceutical Schedule - rather than a qualitative assessment of whether the

circumstances of the patient are exceptional in themselves - is an improvement. The issue is not that the circumstances of the patient are necessarily exceptional in relation to anyone else, but that they are exceptional in relation to the Pharmaceutical Schedule. This is a point you made in your submission, and Pharmac seems to have taken it on board.

3. I think this is a very significant concession from Pharmac, because the very narrow approach they initially seemed to be taking to the exceptionalness of the particular patients' circumstances flowed through to the overly restrictive "Unique Clinical Circumstances" pathway. The most favourable amendment to the prerequisite pathways has been the change in that pathway from Unique Clinical Circumstances to Unusual Clinical Circumstances. We had argued that the Unique Clinical Circumstances pathway had been prescribed so tightly that it was hard to see anyone qualifying. Changing this pathway to Unusual Clinical Circumstances is undoubtedly an improvement, and it follows directly from the clarification by Pharmac of the basis of the "exceptions" relevant to the Exceptional Circumstances Policy.
4. Pharmac has also added the reference to "no other treatment being available" for both the Unusual Clinical Circumstances pathway, and the Urgent Assessment pathway. I expect this criterion would have applied in practice anyway, even under the draft policy prepared for consultation. The addition of this reference to the Unusual Clinical Circumstances and Urgent Assessment pathways adds another argument in favour of providing exceptional circumstance pharmaceuticals, including on a review, and that certainly does not hurt the interests of those with very rare diseases.
5. I think it is also significant that Pharmac is now saying that the Exceptional Circumstances Policy is not necessarily the only way the availability of pharmaceuticals in exceptional circumstances might be dealt with. Pharmac is clearly saying it has a residual discretion beyond the scope of the Pharmaceutical Schedule and the Exceptional Circumstances Policy, and I am not aware that they have said this before. The new Policy does not specify the scope of the residual discretion, or how it might be exercised. However you can see that Pharmac is interested in retaining flexibility, and this must be encouraging for the hard cases where people might not qualify under the standard Exceptional Circumstances Policy.
6. In terms of the interface between the Exceptional Circumstances Policy and the Pharmaceutical Schedule, it is interesting that the Unusual Clinical Circumstances and Urgent Assessment pathways are not available for a pharmaceutical or set of clinical circumstances which have already been assessed for the Pharmaceutical Schedule. This means a pharmaceutical will only be eligible under the Exceptional Circumstances Policy if it has not already

been assessed for the Pharmaceutical Schedule, and a pharmaceutical that has been rejected for the Pharmaceutical Schedule will not be eligible for an Exceptional Circumstances application. Maybe there will be some leeway in relation to the particular clinical circumstances of a named applicant, but this does seem likely to steer the Exceptional Circumstances Policy towards new pharmaceuticals which have not been assessed for the Pharmaceutical Schedule. This may be a good thing from your point of view, although as a matter of logic it limits the scope of the exceptions available.

7. I think the most significant (and disappointing) aspect of the new Exceptional Circumstances Policy is that Pharmac has not made any changes with respect to the assessment process and the nine Decision Criteria. This is also consistent with limiting pharmaceuticals available in exceptional circumstance to those that have not already been assessed for the Pharmaceutical Schedule, and it reinforces the primacy of the Pharmaceutical Schedule in Pharmac's decision-making. Your submission criticised the idea of deciding on exceptions under the same Decision Criteria as apply to Pharmaceutical Schedule decisions, but Pharmac has not accepted that the Decision Criteria should be modified in the case of exceptional circumstances applications. The new Policy does not set out the Decision Criteria, but they are referred to and there is no doubt as to what Pharmac means by those references.
8. The consultation draft of the policy had indicated that Pharmac would modify its assessment criteria for the purposes of the Exceptional Circumstances Policy, and your submission said those modifications did not go far enough when they could have referred to individual rights or ethical considerations, for example. The position Pharmac now seems to have taken is that it will apply a standard and completely unmodified set of the Decision Criteria to Exceptional Circumstances applications.
9. This ties in with the point you have made in relation to the cost-benefit analysis Pharmac has said it will continue to apply to orphan-type medicines, where those medicines will inevitably be at a disadvantage on a cost-benefit basis because of their inherent high cost, and the lack of any economies of scale in their supply.
10. The Decision Criteria are themselves so discretionary that Pharmac will essentially be able to do what it wants at the assessment phase of the Exceptional Circumstances Policy. This is the point in the process where the value judgements will be made, and there is a risk that the flexibility inherent in the Decision Criteria will render the prerequisite eligibility decisions largely academic. It could be that the change, for example, from the Unique to Unusual Clinical Circumstances for the first eligibility pathway reflects a more open approach to exceptional circumstances by Pharmac, but there is a risk

that the underlying reliance on the same Decision Criteria used for the Pharmaceutical Schedule will create the opportunity for Pharmac to frustrate the approach which might otherwise seem to be more open. Hopefully this is not Pharmac's intention.

11. The most positive and constructive part of the Exceptional Circumstances Policy is the funding increase from \$4 million to \$8 million. The policy makes it very clear that this is an annual amount, and this increase is obviously a significant amount. I note that Pharmac has already confirmed to you that there may be an element of double-counting in the increased figure because of the simultaneous combining of three exceptional circumstances schemes into one. Therefore the gain might not be quite as good as it first appears.

The only other comments I have are either about things peripheral to your core interests in this matter, or would match some of the comments you have already made anyway. Hopefully this advice is helpful to you.

Yours sincerely



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