Ombudsman
Fairness for all

Opinion

Ombudsman consideration of Pharmac decision not to approve funding for "Myozyme"

Ombudsman's opinion

Legislation:	Ombudsmen Act 1975, s 22; New Zealand Public Health and Disability Act 2000, ss 47 & 48
Agency:	Pharmaceutical Management Agency (Pharmac)
Ombudsman:	Dr David McGee
Reference number:	343665
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Summary

The complainant suffers from adult late-onset Pompe disease, a metabolic disorder which is one of a number of diseases collectively called lysosomal storage disorders. Myozyme is an enzyme replacement therapy used in the treatment of Pompe disease. There are no alternative medicines available to treat the form of Pompe disease from which the complainant suffers.

In 2011 and 2012, the complainant made applications for a subsidy for Myozyme in exceptional circumstances. Myozyme is not listed on the Pharmaceutical Schedule, and is therefore not publicly funded by Pharmac apart from in exceptional circumstances. Both applications were declined.

Following an investigation of Pharmac's decisions, in this Opinion the Ombudsman notes that in considering the second application, Pharmac should have specifically responded to the suggestion made by a clinician, on behalf of the complainant, of funding for a trial period.

The Ombudsman also examines the criteria for decision making on:

- the Pharmaceutical Schedule, under which listed medicines are publicly funded for any New Zealand-eligible patient; and
- the Named Patient Pharmaceutical Assessment Policy (NPPA Policy), under which applications may be made by individual patients in exceptional circumstances for subsidised access to treatments which are not included on the Pharmaceutical Schedule.

If a treatment has been "*prioritised*" by Pharmac for consideration for listing on the Pharmaceutical Schedule, then this is a factor taken into account in considering applications by individual patients under the NPPA Policy. The Ombudsman comments that Pharmac should more clearly build an external element into its prioritisation decision-making in relation to the Pharmaceutical Schedule, since prioritisation has consequences for funding eligibility under the current NPPA Policy that Pharmac operates.

The Ombudsman further observes that the NPPA Policy itself ought to provide that prioritisation will not be a pre-requisite if substantial new information of a clinical or commercial nature becomes available, and suggests that Pharmac reconsider its terminology of "*prioritisation*" to give a clearer indication of the consequences of an assessment of a medicine and its assignment of a priority for funding.

Finally, the Ombudsman notes that the criteria under the NPPA Policy ought to be clearly differentiated from those under the Pharmaceutical Schedule.

Introduction

1. Ms Evans suffers from adult late-onset Pompe disease, a metabolic disorder which is one of a number of diseases collectively called lysosomal storage disorders. The primary symptom is described as muscle weakness causing mobility problems and respiratory weakness. The condition is not necessarily fatal but premature death is not uncommon. In Ms Evans' case it is accepted that without an effective treatment she will continue to deteriorate and may die prematurely as a result of respiratory failure.

2. Alglucosidase alpha (patented as Myozyme) is an enzyme replacement therapy used in the treatment of Pompe disease. There are no other alternative medicines available to treat the form of Pompe disease from which Ms Evans suffers. Such other treatments as are available are therapies to support the sufferer only.

3. The Pharmaceutical Management Agency (Pharmac) maintains a Pharmaceutical Schedule listing medicines and medical treatments. A medicine's inclusion on this schedule means that that medicine will be publicly funded for any New Zealand-eligible patient for whom it is prescribed, subject to any access criteria set out in the schedule. Myozyme is not listed on the schedule.

4. However, Pharmac is obliged to make provision for subsidising, in exceptional circumstances, medicines not listed on the Pharmaceutical Schedule. In 2011 and 2012 Ms Evans made applications to Pharmac for a subsidy to allow her to be treated with Myozyme under the policies operated by Pharmac at those times for providing such funding. Both applications were declined.

5. On 19 October 2012 Ms Evans, assisted by Lysosomal Diseases New Zealand, the Muscular Dystrophy Association of New Zealand and the New Zealand Organisation for Rare Disorders (NZORD), made a complaint to this Office about Pharmac's decisions. She gave me substantial background material on her own case and on that of another Pompe disease sufferer. Mr John Forman, Executive Director of NZORD, became the principal point of contact for Ms Evans' complaint.

6. Ms Evans' complaint was notified to Pharmac, which provided me with a comprehensive report. I subsequently met with Pharmac's Medical Director, the Team Leader Medical, and its General Counsel, to discuss this report. Following this meeting Pharmac provided me with two further reports responding to issues I had raised. I also met with Mr Forman to discuss the complaint and received further information from him during my consideration of it.

7. On 24 April 2013 I issued my provisional view on the complaint to Ms Evans and Pharmac. I received comments from them both and I have reflected these in this opinion as I considered appropriate. However, one matter arising from a comment I made in my provisional view has required more extensive treatment and I have dealt with it as an addendum at the end of this opinion.

Ombudsman's role

8. The Ombudsmen Act does not prescribe the objects of an Ombudsman's investigation of a complaint under that Act but section 22(1) and (2) set out a number of conclusions that the Ombudsman may draw and which would justify the Ombudsman making a formal recommendation under that section. These conclusions are taken to give a focus to investigations under the Act.

9. Section 22(1) and (2) read:

> *"(1) The provisions of this section shall apply in every case where, after making any investigation under this Act, an Ombudsman is of opinion that the decision, recommendation, act, or omission which was the subject-matter of the investigation-*
>
> *(a) appears to have been contrary to law; or*
>
> *(b) was unreasonable, unjust, oppressive, or improperly discriminatory, or was in accordance with a rule of law or a provision of any Act, regulation, or bylaw or a practice that is or may be unreasonable, unjust, oppressive, or improperly discriminatory; or*
>
> *(c) was based wholly or partly on a mistake of law or fact; or*
>
> *(d) was wrong.*
>
> *(2) The provisions of this section shall also apply in any case where an Ombudsman is of opinion that in the making of the decision or recommendation, or in the doing or omission of the act, a discretionary power has been exercised for an improper purpose or on irrelevant grounds or on the taking into account of irrelevant considerations, or that, in the case of a decision made in the exercise of any discretionary power, reasons should have been given for the decision."*

10. An Ombudsman's investigation is not limited to the reasonableness or correctness of the decision, etc under review. It can also consider the reasonableness of the law or any practice (which I take to include policy) under which that decision was taken. I will discuss this jurisdiction and its limits later in this opinion.

Pharmac

11. In 1993 the then regional health authorities decided to collaborate in the purchasing of medicines so as to improve their purchasing power in dealing with the pharmaceutical industry. For this purpose a limited company was registered called the Pharmaceutical Management Agency Ltd, commonly abbreviated to "*Pharmac*".

12. From 1 January 2001 (by the New Zealand Public Health and Disability Act 2000) this arrangement was brought to an end. The limited company was dissolved and in its place a stand-alone statutory body, the Pharmaceutical Management Agency (also known as Pharmac), was created with a wider set of functions. One of these was determining which medicines would be publicly funded as well as negotiating their prices. On 25 January 2005 Pharmac became a Crown entity (as a Crown agent) following the enactment of the Crown Entities Act 2004.

13. Pharmac's objectives are set out in section 47 of the New Zealand Public Health and Disability Act. These are:

> *"(a) to secure for eligible people in need of pharmaceuticals, the best health outcomes that are reasonably achievable from pharmaceutical treatment and from within the amount of funding provided; and*
>
> *(b) any other objectives it is given by or under any enactment, or authorised to perform by the Minister by written notice to the board of Pharmac after consultation with it."*

14. Section 48 of the Act then elaborates Pharmac's functions in the following terms:

> *"The functions of Pharmac are to perform the following within the amount of funding provided to it and in accordance with its statement of intent (including the statement of forecast service performance) and (subject to section 65) any directions given under the Crown Entities Act 2004:*
>
> *(a) to maintain and manage a pharmaceutical schedule that applies consistently throughout New Zealand, including determining eligibility and criteria for the provision of subsidies:*
>
> *(b) to manage incidental matters arising out of paragraph (a), including in exceptional circumstances providing for subsidies for the supply of pharmaceuticals not on the pharmaceutical schedule:*
>
> *(c) to engage as it sees fit, but within its operational budget, in research to meet the objectives set out in section 47(a):*
>
> *(d) to promote the responsible use of pharmaceuticals:*
>
> *(e) any other functions it is for the time being given by or under any enactment, or authorised to perform by the Minister by written notice to the board of Pharmac after consultation with it."*

15. Ministerial directions requiring Pharmac to purchase a pharmaceutical from a particular source or at a particular price or to provide any pharmaceutical subsidy to a named individual are specifically prohibited (section 65(2)).

16. In regard to Ms Evans' complaint, the most significant functions of Pharmac that are engaged are its responsibility under section 48(a) to maintain and manage a

pharmaceutical schedule and that under section 48(b) to provide, in exceptional circumstances, for subsidies for the supply of pharmaceuticals that are not on the schedule. These are critical functions. Pharmac–approved medicines are funded from a dedicated fund, the Combined Pharmaceutical Budget (CPB), set by the Minister of Health following consultation with DHBs, forecasting by Pharmac, and advice from the Ministry of Health. The CPB is set annually and, as well as funding medicines already on the schedule, funds all medicines that are added to the schedule (or extensions made to the access criteria for those already listed) during the course of the year and any medicines or medical devices approved under the "*exceptional circumstances*" provision of section 48(b). Although the CPB funding is distributed among the DHBs, it is managed centrally by Pharmac.

17. Pharmac emphasises that the CPB is a fixed budget within which it is required to perform its functions. I do not know what the arrangements were when the forerunner of the present Pharmac was established in 1993, but it is certainly true that the New Zealand Public Health and Disability Act makes it explicit that Pharmac is not just concerned with getting the best deal possible in purchasing pharmaceuticals. Pharmac works within a funding ceiling. It is obliged to operate within "*the amount of funding provided*", a phrase that occurs in both section 47 and section 48.

18. However, while the total CPB is fixed its components (that is, amounts spent on scheduled medicines and exceptional circumstances subsidies from within it) are not. The CPB total covers the forecast expenditure on medicines on the schedule, of course, but Pharmac (through a memorandum of understanding with DHBs) also agrees an amount of funding within the CPB for exceptional circumstances subsidies (which may relate to treatment by existing or new medicines). Pharmac monitors such spending to ensure that it does not lead to over-expenditure of the total CPB. In making its "*exceptional circumstances*" decisions Pharmac is conscious of the overall budgetary impact of those decisions.

Named Patient Pharmaceutical Assessment Policy

19. To carry out its function of providing for subsidies in exceptional circumstances, Pharmac has developed a formal policy. The current policy is called the Named Patient Pharmaceutical Assessment Policy (NPPA Policy). It was introduced with effect in March 2012. This policy is concerned with applications from individual patients for subsidised access to treatments for their particular clinical circumstances where such treatments are not funded under the Pharmaceutical Schedule on a population basis.

20. Consideration of Ms Evans' applications for funding straddle both the NPPA Policy and the previous Exceptional Circumstances Scheme (EC Scheme). The move from the EC Scheme to the NPPA Policy involved a detailed consideration by a review panel of the EC Scheme and other public input and comment on that policy, and then a new proposed policy (which eventually became the NPPA Policy) being issued for full public consultation

and feedback. In this way Pharmac set out to comply with its statutory obligations to consult on such matters (as required by section 49).

21. I have heard no criticism of the consultation process followed by Pharmac in developing the NPPA Policy. There has certainly been criticism of elements of that policy and how Pharmac has applied those elements. However, I acknowledge as significant the point made by Pharmac that the NPPA Policy was only adopted after a comprehensive consultation exercise.

22. The NPPA Policy provides for three different "*pathways*" to exceptional circumstances funding, each with different pre-requisite requirements. In Ms Evans' case, Pharmac applied one of these - the Urgent Assessment (UA) pathway. This is intended to deal with patients whose condition may not be very unusual but who may be particularly disadvantaged if required to wait until a new medicine is considered for funding by a listing on the Pharmaceutical Schedule.

23. Ms Evans told me that she was surprised that her application has been assessed under the UA pathway. But she accepted that the likely alternative pathway under the NPPA Policy did not fully suit her circumstances either. One of the UA pathway's pre-requisite requirements is that the treatment has not been "*prioritised*" by Pharmac for consideration for listing on the Pharmaceutical Schedule. Myozyme had been prioritised by Pharmac following an unsuccessful application for funding by another patient under the EC Scheme. In these circumstances Ms Evans did not meet one of the pre-requisites for UA.

24. I will come to the implications of "prioritisation" below but, given that this requirement of the applicable pathway under the NPPA Policy meant that her application must fail, I raised the question of whether Ms Evans might not have been disadvantaged by the move from the EC Scheme to the NPPA Policy. Under the EC Scheme her clinical circumstances meant that she met its pre-requisites and she would not have had to rely on Pharmac exercising an overriding discretion in order to be considered.

25. In response Pharmac told me that the EC Scheme did not cover "*in-hospital*" treatments such as infusions (which Myozyme is). Therefore, it said, Ms Evans did not meet the requirements of the EC Scheme either in this regard. Nevertheless, Pharmac had exercised its discretion to consider Ms Evans' application on its merits but had still decided to decline to provide funding. Pharmac did concede that in its letter of 20 September 2012 to Ms Evans' clinician it had not clearly explained the process that it had followed. It should have made it clearer that, despite the NPPA Policy pre-requisite not having been met, Pharmac had actually gone on to consider the application and that the decision to decline had been made after applying (in Pharmac's view) the nine decision criteria specified in the policy. Whether it did the latter correctly is a particular aspect of Ms Evans' complaint.

Prioritisation status of Myozyme

26. As stated above, the reason given for deciding that Ms Evans' application under the NPPA Policy did not meet a pre-requisite was that Myozyme as a treatment for Pompe disease had been placed on Pharmac's prioritisation list for inclusion on the Pharmaceutical Schedule. It is a pre-requisite for funding under the UA pathway of the NPPA Policy that this has not occurred.

27. As I have indicated, Pharmac did give more consideration to Ms Evans' application than the fact of its prioritisation and relied on other grounds for its decision to decline the application. Nevertheless, I queried why "*prioritisation*" should be an effective stop on applications under the NPPA Policy, at least as far as the UA pathway was concerned.

28. Pharmac explained that its "*assessment*" of each medicine takes place before prioritisation. The reason for a pre-requisite of this nature is that where applications for funding have been fully assessed they take their place among other fully assessed applications awaiting the availability of funding on suitable commercial terms and competing, in effect, with those other fully assessed applications in accordance with the level of priority assigned to them. When a medicine has been prioritised whether it will be funded depends on the amount of funding available in the year and Pharmac's ability to negotiate a suitable commercial arrangement with the supplier.

29. In Myozyme's case it sits on Pharmac's prioritisation list as an "*option recommended for decline*". It ranks equal last with approximately 80 other funding applications also recommended for decline. No active work is undertaken on any medicines sitting under this category. However, Pharmac says it is able to reassess and analyse an application for Myozyme in the event that new information (clinical or commercial) is provided about it.

30. The first question I raised with Pharmac was whether placing a medicine on the prioritisation list could be seen as a convenient means of avoiding having to consider a NPPA Policy funding application. If the medicine has been prioritised, a pre-requisite for NPPA Policy funding has not been met and the application can be declined at that point. (I add that this did not occur in the case of Myozyme. Ms Evans' application proceeded to full consideration despite the prioritisation.) This, it seemed to me, could set up an undesirable incentive to prioritise medicines.

31. In response to my concerns Pharmac emphasised the elaborate process that it follows before a medicine can be prioritised. This involves consideration of draft prioritisation lists with considerable background information at meetings within Pharmac held three or four times a year. (I have set out Pharmac's prioritisation process as described to me in an annex to this opinion.) It is clear that a prioritisation decision is not a casual one and that there is considerable internal input into it. As I have said, I make no suggestion that the process has been abused or misused.

32. But, that said, a prioritisation decision is now a most significant one in terms of the NPPA Policy (at least, under the UA pathway). Subject to an overriding discretion by Pharmac (exercised in Ms Evans' case), it removes a medicine from the possibility of being funded

under that policy. In my view, the importance of a prioritisation listing for the NPPA Policy having been elevated in this way raises the question of building an external element into the decision to prioritise in the first place.

33. I note that the prioritisation meetings are arranged to coincide with meetings of the Pharmacology and Therapeutic Advisory Committee (PTAC - a committee Pharmac is obliged to establish under section 50(1)(a) of the New Zealand Public Health and Disability Act). That committee has an outside membership. Pharmac told me, in response to my provisional view, that comment from PTAC is invited following the internal prioritisation process described in the annex. Nevertheless its involvement in prioritisation decisions could perhaps be made more explicit.

34. The next issue I have considered is the consequence of prioritisation for exceptional circumstances funding.

35. "*Prioritisation*" as a term does seem to me to convey the impression of some priority (early consideration or importance) for inclusion on the schedule, though I accept that prioritisation is actually a neutral term. It can lead to a low priority as well as a high priority being assigned. But if all Pharmac means by "*prioritising*" a medicine is that that medicine has been assessed and a priority assigned to it, I question why a more straightforward description of where such a medicine stands in relation to the schedule is not employed.

36. The UA pathway under the NPPA Policy requires as a pre-requisite that the medicine applied for has not been fully assessed by Pharmac. If it has been assessed and assigned a priority (high or low) it will not be available under the NPPA Policy.

37. In its response Pharmac told me that Myozyme, for example, could be reassessed in the event that new information about it becomes available. I accept that Pharmac has the discretion to reassess Myozyme and the other medicines on the prioritisation list, but the NPPA Policy does not recognise that this may occur. As far as the NPPA Policy is concerned if a medicine has been "*prioritised*" it is not available under the UA pathway of that policy. There is nothing in the policy about considering an application for an already prioritised medicine where new information is available. Pharmac has told me that where new information comes to light about a prioritised medicine, it would generally review the earlier funding application, rather than take up that information as part of a new NPPA Policy application and disregard the pre-requisite. This is very well as far as it goes but it still leaves everything on the basis of a general discretion. I do not think that this is satisfactory.

38. I agree that Pharmac will not wish to go through further assessment processes for numerous NPPA Policy applications for the same medicine. But it is reasonable to expect Pharmac to reassess a medicine if new information about it becomes available. (Ms Evans believes that further information on Myozyme has become available.) In these circumstances it seems to me that the NPPA Policy should expressly recognise this. What is new information sufficient to overcome the pre-requisite bar to consideration is, of course, a matter of judgment in each case. But what is important is that Pharmac

would be making a judgment (against defined criteria) to reopen consideration under the NPPA Policy, rather than merely exercising a discretion at large to do so.

Assessing individuals versus populations

39. As already noted, the NPPA Policy is a means of funding treatment for an individual, named, patient. Funding for a population group is provided through a listing on the Pharmaceutical Schedule. This leads to some issues on the relationship between the two schemes.

40. If all patients with the same complaint were able to obtain funding for a particular treatment under the NPPA Policy this would suggest that that treatment ought to be listed on the schedule as being available regardless of the identity of the patient (though subject to any clinical conditions specified in the schedule for access to that treatment). On the other hand, it would appear inequitable if, of two patients with the same clinical condition, one was approved for treatment under the NPPA Policy and the other was rejected. Pharmac attempts to avoid anomalies and inequities, as far as possible, by treating like cases alike. This is an appropriate objective.

41. Ms Evans emphasises, in addition to clinical conditions, the importance of entry criteria. For example, of patients with the same clinical condition, some might have access to a clinical trial and others might not. An entry criterion in these circumstances might, for example, specify funding for those without access to a trial. While this is an example where there may be a clear distinction between clinical conditions and entry criteria, I am not sure that it is always possible to distinguish between the two. Another example suggested was level of disease progression, which seems to me to relate to clinical condition rather than to be distinctly an entry criterion. In defining the clinical conditions attracting subsidy, Pharmac is in effect adopting entry criteria.

42. I was interested to explore how Pharmac's need to act equitably had impacted on its assessment of Ms Evans' applications. One means employed by Pharmac – the 'prioritisation' of Myozyme for consideration for the schedule – I have already discussed. Clearly, one way of meeting needs for all those suffering adult late-onset Pompe disease would be to fund a suitable treatment on the schedule.

43. But that is not the case at present and the extent to which accepting Myozyme for funding in Ms Evans' case would have implications for funding other sufferers of the disease under the NPPA Policy is a matter that has arisen during Pharmac's consideration of her case.

44. Ms Evans' herself gave me details of an earlier application by another patient for funding for Myozyme under the EC Scheme. This application was declined and the reasoning in the decision on Ms Evans' application a few months later drew extensively on the consideration of that case. A Pharmac paper in September 2011 estimated that there were 50-70 people who had been born with late-onset disease and had been diagnosed with it or were not yet old enough to have been diagnosed but were living with it as a

potential emergent condition. The number actually identified with a similar form of the disease to Ms Evans was much fewer than that. At that time Pharmac knew of eight persons. It is clear that the potential budgetary effects if all persons known to be suffering from adult late-onset Pompe disease sought funding for treatment with Myozyme is a major concern for Pharmac. (Some of the known sufferers, including the earlier patient who was declined in 2011, are understood to have been included in a clinical trial of another medicine not yet approved for use in New Zealand. Nevertheless, there is still the budgetary implication that, if an application was accepted under the NPPA Policy, they may leave the trial and seek funding under the NPPA Policy themselves.)

45. Pharmac's analysis of Ms Evans' EC Scheme application emphasised that its recommendation to decline her application would not change if she was the only patient with her condition. In Pharmac's view the cost of treatment compared to the likelihood of benefit still did not justify its funding.

46. Although the NPPA Policy is a named-patient policy, the fact that there are other patients with the same clinical condition as the applicant is not an automatic disqualification. The UA pathway explicitly recognises that it is available in respect of applications received after Pharmac starts to consider a listing on the schedule if before this Pharmac has funded any patient with the same clinical circumstances. Indeed the potential clinical circumstance of a population group is a pre-requisite for funding under that pathway. So the NPPA Policy does not just apply to an individual with a unique clinical condition.

47. Despite this, each application is assessed on an individual basis and this can potentially lead to patients with the same or similar clinical conditions having a different outcome from their applications. The clinical data about a particular treatment may change over time (suggesting that it is more or less effective) and the treatment cost may change (becoming more or less expensive). These factors will alter the assessment under the policy at different times, even where a later patient has the same clinical conditions as an earlier one.

48. In Ms Evans' case Pharmac used the limited population data it had available to it to assess Myozyme's likely effectiveness in her case on the basis that she was in a similar circumstance to the population group about whom there was data. It then adjusted for her age and weight. As its reconsiderations of her applications progressed it adjusted these calculations. Its last assessment of Ms Evans' weight was at a lower and, from the point of view of approval of her application, more favourable level than its earlier one.

49. In responding to my request to reconsider Ms Evans' position on the basis of the latest weight she had provided (referred to below), Pharmac went on to say this:

> *"As you know, we consider that if we are making funding available for an individual we should also make the treatment available to other patients with the same or similar condition. Therefore any change in Ms Evans' weight would have little effect on the assessment of the impact of approving alglucosidase alpha (Myozyme) for all patients suffering from the same*

> *condition (i.e. the wider group). For treatments that are dosed by weight, we note that approving treatment for a light patient while declining treatment for a heavy patient could arguably be unlawful discrimination (given that there are a number of reasons why one person might be heavier than another including sex, race, age) and could incentivise a heavier person to, detrimentally, lose weight."*

50. Ms Evans considers that Pharmac is overstating the case here. She instances other examples in the health system where "*discrimination*" occurs, such as admonitions to lose weight before surgery.

51. Without expressing a view on whether there is a legal issue involved, I do not take issue with the general sentiments expressed by Pharmac. Equity does demand equality of access to treatment. But it does suggest to me that the implications for others of funding Ms Evans under the NPPA Policy do weigh heavily with Pharmac. If even material changes in Ms Evans' individual circumstances (her weight) have very little impact on Pharmac's judgment of the cost-effectiveness of the proposed treatment, this implies a stronger emphasis on population or group considerations than may be understood from a "named-patient" pharmaceutical assessment. This strengthens the view that there is a close relationship between applications under the NPPA Policy and listings on the Pharmaceutical Schedule, a matter to which I will return.

Decision Criteria

52. The NPPA Policy (sections 4e and 4f) states that Pharmac will assess applications made under the policy according to what are described as nine Decision Criteria. These nine criteria are those adopted by Pharmac for making assessments of pharmaceuticals for inclusion on the Pharmaceutical Schedule. No question has been raised about Pharmac's reliance on the first seven of these criteria. But Ms Evans criticises how Pharmac applied criteria 8 and 9 to her application. These criteria read:

> *"8. the Government's priorities for health funding, as set out in any objectives notified by the Crown to PHARMAC, or in PHARMAC's Funding Agreement, or elsewhere; and*
>
> *9. such other criteria as PHARMAC thinks fit."*

53. In a memorandum to the Pharmac Directors in September 2011 on Ms Evans' EC Scheme proposal, Pharmac said, in respect of criterion 8, "*No such objectives are relevant to assessing this proposal*" and, in respect of criterion 9, "*No other criteria are relevant to assessing this proposal*". These are the same responses that were included in a memorandum of February 2011 reporting to the Board on an earlier application by another patient for funding for Myozyme.

Criterion 9 – Other criteria

54. In its response to me Pharmac pointed out that neither Ms Evans nor her clinician provided it with information or comments on these two decision criteria. This is a relevant observation. But it then went on to say that even if such information had been provided Pharmac's Operating Policies and Procedures state that Pharmac will carry out appropriate consultation when it intends to take such "*other criteria*" into account. If additional matters were to be considered by Pharmac, as contemplated by criterion 9, Pharmac considers that it would have been obliged to consult on any proposal to do so.

55. Consultation as a matter of principle is an eminently desirable thing to do and, of course, such consultation as is carried out does not need to be of an "*at large*" nature. It can be tailored to what is appropriate in the circumstances.

56. Pharmac's Operating Policies and Procedures to which it (but not the NPPA Policy) refers (in para 2.2) list the nine Decision Criteria. Under the ninth (identified in that document as (i)) it is explained that "*PHARMAC will carry out appropriate consultation when it intends to take any such 'other criteria' into account*". But crucially the Decision Criteria in this document refer to how Pharmac goes about considering amendments to the Pharmaceutical Schedule (that is the actual heading of that part of the document). I agree at once that decisions of that nature should be made on the basis of criteria consulted on and advertised in advance. The arguments of NZORD and other organisations have been that social and ethical factors should be explicitly recognised in the Decision Criteria. Pharmac's position is that they are recognised, at least implicitly, in the legislation. It does not see criterion 9 as a means of importing such factors into consideration.

57. It is, of course, always open to Pharmac to alter from time to time its Decision Criteria regarding inclusion on the Pharmaceutical Schedule by adding a new criterion or new criteria after consulting on them. Criterion 9 would thus appear to add nothing substantive to what Pharmac might do anyway (except to make this explicit). Criterion 9, on this view, appears to be contentless unless and until Pharmac has consulted on a new criterion and agreed to include it. But I am not concerned here with the Pharmaceutical Schedule, I am concerned with the NPPA Policy.

58. The Operating Policies and Procedures do say that where Pharmac makes other decisions (it instances its demand side activities), "*it endeavours to use [the Decision Criteria] to the extent that they can be applied to those decisions*". No doubt those criteria can appropriately be applied to NPPA Policy decisions as section 4f of that policy contemplates. But an assessment for inclusion on the Pharmaceutical Schedule (with its general implications) and approval under the NPPA Policy (with its more limited effects) are different things.

59. Ms Evans claims that "*Pharmac is trying to shoe-horn all exceptional circumstances applications into a single decision making checklist, based on the same criteria as a Schedule application*" (letter of 19 October 2012). I was therefore interested in the

consultation that Pharmac would feel obliged to engage in under criterion 9 in an NPPA Policy context as opposed to a Pharmaceutical Schedule context.

60. Pharmac emphasised to me that, even apart from the Decision Criteria, it retains the discretion to consider applications for funding outside the NPPA Policy and that it has in fact exercised this discretion. I agree, and I see criterion 9 as (at least in part) reflecting this discretion. Notwithstanding the specific factors identified in criteria 1 to 8, Pharmac reserves to itself the right to take other factors into account and fund treatment if it sees fit. This is an appropriate position to take. But it would be unreasonable if, in making a decision on an NPPA Policy application, Pharmac would not even consider factors (criteria) suggested to it that fell outside those identified in criteria 1 to 8 without first engaging in a full round of consultation as if an amendment to the schedule were under consideration.

61. In specifically responding to what consultation might have been required in this case had submissions falling into the "*other criteria*" category been made to it, Pharmac remarked that it had been unable to identify an instance when this criterion had been used. It considered that if such a factor had been accepted this would probably have led to a permanent amendment to the Decision Criteria. Pharmac also emphasised that it would consider whether any other factors suggested to it fell within its statutory objective before taking them further. It instanced factors that did not relate to health outcomes, since it considered that its statutory objective required it to focus on health outcomes only.

62. If other factors were suggested to it which it felt were relevant it would consult with potentially affected members of the public, groups or individuals. It would identify affected stakeholders by the nature of the proposal itself.

63. I am satisfied that Pharmac would follow a consultation process appropriate to the application at hand if other factors were suggested to it that engaged the "other criteria" factor. But I do think that the present cross-reference approach adopted by Pharmac for defining the relevant factors for consideration under the NPPA Policy contributes to an impression that applications for exceptional circumstances funding are treated as bids for inclusion on the Pharmaceutical Schedule. There may well be a strong relationship between the two but they are not the same. Pharmac may wish to give further consideration to how it invokes the Decision Criteria for NPPA Policy applications to avoid this impression arising.

64. In this case no submissions were made to Pharmac about the Decision Criteria before it took the decisions on Ms Evans' applications. In this regard Ms Evans says that the application forms and process do not clearly invite applicants to structure the application against the specific Decision Criteria. That is a pertinent observation that may require Pharmac's attention. It may have arisen because of the unduly complicated way in which those criteria are imported into the NPPA Policy. But in this case it is clear that Pharmac has not unreasonably failed to consider other grounds (criteria) that were suggested to it. Criterion 9 (the residual discretion criterion) did not, as such, arise for consideration at all at the time Pharmac made its decision. The matters that Ms Evans now urges for

consideration are matters which she would wish to see included explicitly in the NPPA Policy and I will consider them in that context.

Criterion 8 – Crown objectives

65. As far as criterion 8 is concerned, since Pharmac did not receive any submissions on it the only question is whether it was reasonable for Pharmac itself to conclude that there were no objectives notified by the Crown to Pharmac or in Pharmac's Funding Agreement or elsewhere that were relevant to the application.

66. Ms Evans suggests that Pharmac had not considered that the Government has indicated that the need to improve access to highly specialised medicines is a priority area of focus. She specified two examples –

    a. New Zealand's Medicine Strategy 2007 and Actioning Medicines New Zealand (updated and released by the Associate Minister of Health in 2010) emphasised the principle of equity and acknowledged work that the Minister of Health had initiated to improve access to high-cost, highly specialised medicines;

    b. the Government's 2010/11 Letter of Expectations to Pharmac expected that Pharmac would respond to specific expectations and areas of focus including: "*Achieving better access to medicines, which will include continuing to work with [the Minister] and key stakeholders around improving access to high cost, highly specialist medicines*".

67. In response, Pharmac referred me to advice it had received from the Ministry of Health to have regard to the targets identified in its Statement of Intent (for 2012 through to 2015) and to the fact that the Minister of Health's latest Letter of Expectations for 2012-13 had recently been placed on Pharmac's website. This set out the Minister's expectation that the NPPA Policy "*needs to improve access to high cost, highly specialised medicines*".

68. It is clear that Pharmac was well aware of the Government's health priorities when it made its decisions, indeed these are a background to all Pharmac decisions. It is also true, as Pharmac said to me, that the Minister's expectations (or indeed other priorities expressed necessarily in fairly general terms) do not create an obligation to fund every application for high cost, specialised medicines. The NPPA Policy is intended to provide the framework for such decisions and Pharmac contends that it has improved access. It claims that application numbers under the new policy are up 16% compared with the former EC Scheme and the approval rate for NPPA Policy applications is about 68% compared with 40% previously. It says that some very expensive and highly specialised treatments have been funded under the NPPA Policy, including enzyme replacement therapies for some lysosomal storage disorders.

69. Pharmac's report on criterion 8 in Ms Evans' case is fairly brusque. This criterion should not become just a box to tick. If that was the case it should not be there. But I am

satisfied that Pharmac is well aware of and takes into account the relevant health priorities when making its decisions.

Other NPPA Policy criteria

70. Ms Evans advanced a number of criticisms of Pharmac's exceptional circumstances decision-making based on criterion 9, the residual criterion. I have referred to this criterion above when considering the Decision Criteria generally. As I indicated above I do not accept that its use is as limited as when a proposal for inclusion on the Pharmaceutical Schedule is under consideration. Specifically, there should not be such a high consultation threshold for invoking other factors when reaching decisions under the NPPA Policy as there must be when deciding on Pharmaceutical Schedule listings.

71. In summary the criticisms advanced by Ms Evans that I discuss below are as follows:

    a. departure from cost-effective thresholds;

    b. applying "*community values*";

    c. difficulties with rare diseases.

Cost-effectiveness

72. Ms Evans put it to me that in an exceptional circumstances application Pharmac must consider what criteria make that application exceptional and then whether those exceptional factors (together with the usual criteria) justify funding. I have referred above to the factors which Ms Evans considers make her application exceptional. In these circumstances Pharmac should be prepared (she says) to depart from the strict cost-effectiveness analysis applicable when deciding to list medicines on the Pharmaceutical Schedule.

73. I have already noted that there is a strong emphasis in the legislation on the amount of funding available for pharmaceutical treatment. (I will say something more about this later in this opinion.) Pharmac makes the point to me that there will always be a greater demand for funded medicines than the available resources allow. Its nine Decision Criteria (applied to both schedule and exceptional circumstances assessments) are intended to provide a robust process that funds medicines within the budget that is allocated so as to produce the best health outcomes possible from that budget.

74. While there is undoubtedly a heavy (perhaps predominant) element of cost-effectiveness analysis involved in Pharmac's processes, the Decision Criteria are not exclusively concerned with this. The first two are high-level considerations relating to the health needs of the New Zealand population and particular components of that population. Criterion 3 raises a threshold factor – what alternative treatments are available? Criterion 8 (discussed above) relates to the Government's health objectives as communicated to Pharmac.

75. Criterion 4 (what are the likely clinical benefits), criterion 5 (which is explicitly concerned with cost-effectiveness), criterion 6 (the budgetary impact), and criterion 7 (funding contributed by the health user himself or herself) do go to the cost-effectiveness assessment of the proposed medicines. But, while there is a strong weighting to this factor, it is not the exclusive concern of the Decision Criteria. These do require and permit other factors to influence the final decision.

76. Furthermore, Pharmac emphasises that it always retains the ability to fund a medicine even though it might not meet the tests prescribed by the Decision Criteria. It has done this on occasion (as described above).

77. I am satisfied that a balance has been struck by Pharmac between a strong attention to cost-effectiveness and other factors which may cause these to be overridden or set aside in an individual case. I appreciate that to talk of cost-effectiveness in this area can appear callous and uncaring. But I do not see any way to avoid it and it is clear that the legislation expects it. I will come back to this later. But if there is a problem, it is the one that I have already discussed – that the NPPA Policy does not itself record the Decision Criteria to be used for decisions under that policy. It incorporates these by reference to another policy (the Operational Policies and Procedures Policy) that was designed specifically for decision-making in regard to the Pharmaceutical Schedule. As I have indicated, I consider that Pharmac should more clearly differentiate between the two.

Applying community values

78. Under this head, Ms Evans urges a number of points.

79. She says that most OECD countries fund Myozyme. This approach is said to be a result of fairness, human rights to medical treatment, and equity of access to medication. Emphasis was placed on a principle of states not abandoning their citizens, a principle of obvious relevance where there is no known alternative method of treatment. Ms Evans suggested that these community values supported her having a fair go on Myozyme - that is, a funded trial. I will discuss this latter suggestion separately.

80. Pharmac in response said that the values that Ms Evans referred to had been suggested during the consultation process that led to the adoption of the present NPPA Policy. Pharmac had responded to these submissions in a paper it issued in June 2011 and which was thus part of the policy-making process that led to the new policy. Pharmac's view (affirmed to me) was that societal values are reflected in the legislative framework, Pharmac acts ethically within that framework and therefore there has been no need to include societal values expressly in the NPPA Policy. Furthermore, whether Myozyme is funded in most OECD countries, at least in a way that is directly comparable with New Zealand's system of funding healthcare, is a difficult matter to establish. I have not attempted to do so.

81. I would observe that even if the legislative framework does reflect the values urged by Ms Evans, that does not preclude them being referred to in policies that Pharmac develops. But I do not intend to engage in second-guessing decisions that were taken in

2011 after an extensive round of consultation. There is no question of unethical behaviour by Pharmac. Pharmac maintains that the exceptional circumstances policy that it has developed – the NPPA Policy – is consistent with its legislative mandate. I have no reason to conclude that it is not, though I appreciate that Ms Evans and her supporters see section 48(b) of the New Zealand Public Health and Disability Act as having a much broader application. Underlying this, I think, is a fundamental disagreement over the approach to funding pharmaceuticals. I will refer to this again below.

Rare diseases and treatments

82. In her submission Ms Evans raises the question of the difficulty, in the case of rare diseases, of gathering clinical information and comparative drug manufacture costs. In her letter to me Ms Evans said:

> *"It is a struggle for any highly specialised medicine to deliver cost-effectiveness and make it onto the Schedule. In the case of Pompe there are such small patient numbers worldwide that it is both difficult to get statistically robust clinical benefit information and the costs are comparatively high (as the pharmaceutical company is seeking to recoup drug development costs from a small number of patients). As a result, there is inherent inequity of access issues for patients suffering from rare disorders (such as Pompe)."*

83. Although this statement is directed to the difficulty of obtaining sufficient data for assessment for inclusion on the Pharmaceutical Schedule (with which I am not concerned), comparable difficulties arise for assessments under the NPPA Policy and the two are interrelated. I think that it is accepted that adult late-onset Pompe disease and Myozyme would fall into the category of rare disease and specialised medicine. Pharmac knows of eight cases of the disease in New Zealand and Myozyme is the only brand of this medicine available.

84. Pharmac told me that the issue of recognising rare diseases had arisen during the consultation process for the NPPA Policy. Pharmac had concluded at that time that it could not target the pre-requisites for individual assessment so as to cover rare diseases specifically. This was because it believed that there was no robust rationale for treating pharmaceuticals for rare diseases differently from other medicines. An important consideration for Pharmac was that to attempt a specific recognition for rare diseases in the NPPA Policy would significantly undermine the Pharmaceutical Schedule process.

85. Pharmac points out that there is no one standard of evidence. The standard is different in all cases, even between different schedule listing applications. For instance, not everything that is funded has been the subject of a randomised control trial. The assessment under its Decision Criteria allows Pharmac to consider the potential for benefit against the 'risk' that greater benefit elsewhere would be foregone.

86. I think that the last point that Pharmac makes is a point of general application. Given the legislative injunction to work within a fixed budget, this is a legitimate concern for

Pharmac. But I do not see it as specifically answering Ms Evans' point. The lack of evidence in regard to rare diseases and treatments inevitably puts their assessment at a potential disadvantage. I say 'potential' because (depending on Pharmac's approach) this could lead to a decision to fund a medicine whose benefits in reality are not as great as other treatments which are declined funding on the basis of more conclusive data. Furthermore, Ms Evans considers that if Pharmac has concluded that it cannot target rare diseases specifically, an important aim of the review which led to the NPPA Policy has not been achieved.

87. I do not think that I can express a view on the merits of this issue. The stance that Pharmac has taken arising from the submissions that it received when consulting on what became the NPPA Policy is not unreasonable, much less wrong. If it were to be changed that would only be following a new round of consultation as contemplated in its Operating Policies and Procedures and section 49 of the 2000 Act.

88. However, one issue specific to Ms Evans' case was raised in her submission. This was whether Myozyme should be funded for a trial period for her.

Funding for a trial period

89. In Pharmac's assessment of Ms Evans' application under the EC Scheme (annexed to a memorandum to the Board in September 2011) it conceded that Ms Evans' high health need gave some justification for funding this treatment ahead of patients with other conditions. However, Pharmac considered that this was insufficient to outweigh the lack of significant clinical benefits and the poor cost effectiveness of the treatment (as seen by Pharmac) and the budget impacts beyond years one and two. Pharmac said:

> *"It would be wholly inappropriate to commence funding a treatment in circumstances where continuing treatment could not realistically be provided in the future."*

90. Ms Evans takes issue with that position. She acknowledges that it will be difficult to confirm how she would react to the medicine. But, she says, she should have a "*fair go*" on the medication for a trial period and her funding be reassessed once her clinical reaction is better understood. In submissions made to Pharmac on Ms Evans' NPPA Policy application her clinician referred to a published paper on treatment that had been based on a 36-month follow-up. She emphasised that in Ms Evans' case 36-months of treatment was not being sought. Instead she proposed treatment for an agreed period (she referred to Australian guidelines suggesting discontinuance after 12 months in the absence of observable improvement or stabilisation) and review and assessment against identified criteria before deciding whether to continue or withdraw treatment. (Ms Evans has since told me that she would like 24 months funded access with periodic 6-monthly reviews.)

91. In response, Pharmac confirmed the initial decision to decline funding. But I cannot see that this particular suggestion from Ms Evans' clinician was specifically addressed again

(though it was drawn to the attention of the advisory panel). This was on the basis, I presume, that Pharmac's position was expressed in the earlier memorandum on her EC Scheme application (when a period of funding for up to two years had been suggested).

92. Funding on the basis suggested in Ms Evans' application is possible as confirmed by Pharmac in a letter to Mr Forman (31 October 2012). In that letter Pharmac said:

> *"In some instances where evidence has been lacking, we approved funding for patients for a period of time with renewal criteria, effectively funding a trial for the patient. We will continue to maintain this as an option. We consider the test of whether a medicine is innovative or not is in how much benefit it provides to the patient."*

93. This position is reflected in para 4L of the NPPA Policy.

94. I raised with Pharmac the suggestion of funding for a trial period and reassessing when Ms Evans' clinical reaction was better understood.

95. Pharmac's response to this was:

> *"Almost all NPPA approvals are given for a fixed time period; after which the applicant is required to make a renewal application. Generally this is because the treating clinician has applied only for a fixed period of treatment (after which it is anticipated there would be no clinical need for on-going treatment).*
>
> *Some NPPA applications are approved as a 'trial' to test whether the treatment results in significant and measurable clinical gains to the particular patient. Such 'trials' specify clear exit and renewal criteria in circumstances where the health benefits to a patient are measurable in ways that are clear, objective and unequivocal. In Ms Evans' case, it would be very difficult to identify such criteria.*
>
> *As we discussed [in Pharmac's meeting with me], there are a range of potential health outcomes from medical treatments – these can include a dramatic improvement, a mild improvement, or stabilisation. We note that, in her letter of complaint, Ms Evans advises that for some patients using alglucosidase alfa (Myozyme) "there is a mild improvement or stabilisation (which is still a significant improvement over the further deterioration that would have occurred without medication)" and "it is hard to measure how successful the medication is, as it is hard to know how much a patient would have deteriorated if they were not taking Myozyme".*
>
> *The potential benefit from treatment with alglucosidase alfa (Myozyme) assumed by PHARMAC considering Ms Evans' NPPA application was based on the upper end of clinical outcomes that have been found in clinical trials, which show only a modest benefit. Even with optimistic assumptions about the size of the potential benefit, the very high cost of the treatment means*

> *that the overall value for money of the treatment is much lower than for other NPPA and Schedule applications available for funding.*
>
> *We do not consider that a trial period would be appropriate given our conclusion that treatment with alglucosidase alfa (Myozyme) would only likely provide at best but a modest benefit (if any), and that significant health benefits that would be forgone by funding it rather than other NPPA and Schedule applications [sic].”*

96. Pharmac has to make highly invidious decisions on the basis of contestable assumptions as to clinical benefit and (to a lesser extent) cost. I am unable to substitute my, necessarily inexpert judgment, for assessments that it has properly made. Pharmac’s position on funding Myozyme for a trial period is a reasoned one, though there are likely to be other views on the robustness of its position.

97. However, I do consider that Pharmac in replying to the submissions made to it by Ms Evans’ clinician should have specifically responded to her suggestion of a limited period, rather than (as appears to have been the case) relied on a statement it made a year earlier as representing its position. At my instigation, Pharmac has set out why it considers that a trial period’s funding is not appropriate in this case but, in my view, it is unsatisfactory that this matter was not addressed specifically when the decision on Ms Evans’ NPPA Policy application was confirmed. Pharmac’s reasons having now been disclosed I consider that it should consider any submissions that Ms Evans wishes to address to it on them.

Cost of treatment

98. In her letter to me Ms Evans questioned Pharmac’s estimate of $654,000 per year as the cost of her treatment. That was based on a weight of 55kg. Her weight had reduced in the past year and was currently 42kg, suggesting lower treatment costs. She suggested a figure of $330,000 per year as her cost of treatment. I asked Pharmac to comment on this.

99. In response Pharmac said that Ms Evans’ subsequently reduced weight is not relevant to any assessment of the reasonableness of Pharmac’s decision at the time it was made. I accept this.

100. Nevertheless, Pharmac did provide a response based on Ms Evans’ currently advised weight, which is only 4.8kg lighter than when Pharmac last considered her NPPA Policy application. Pharmac advises that Ms Evans’ current weight would reduce the cost of her treatment with Myozyme and thus improve the cost-effectiveness of the treatment by approximately 10%. However, it pointed out that its cost-utility analysis for results with Myozyme were varied over a greater range than this. Depending upon the assumptions one made, the cost of treatment could vary down by as much as 75%. Pharmac’s view is that even with optimistic assumptions about the size of potential benefits, the very high cost of Myozyme means that the overall value for money of the treatment even at the

reduced weight is much lower than for other NPPA Policy and Pharmaceutical Schedule options.

Consultation with the Counties-Manukau DHB

101. Ms Evans resides within the area serviced by the Counties-Manukau DHB. While considering Ms Evans' application under the EC Scheme in 2011, Pharmac asked the DHB whether it had any comments to make on the application. In the event, no comment was received from the Counties-Manukau DHB.

102. I queried Pharmac as to why it had asked for comments from the DHB on Ms Evans' application.

103. Pharmac told me that the budget arrangements within the CPB for exceptional circumstances were changed with effect on 1 July 2011. Prior to that date, DHBs set aside a separate 'risk pool' of funds drawn from the CPB to cover expenditure on exceptional circumstances. Contributions to the risk pool were allocated on a population basis so that each DHB knew, in advance, the maximum amount it would be charged for approvals under the EC Scheme during a particular year. On 1 July 2011 this system was changed and expenditure on each patient was charged direct to the relevant DHB.

104. Pharmac received Ms Evans' application while the 'risk pool' arrangements were in place, though the application was not finalised until September 2011. Counties-Manukau DHB (and other DHBs in respect of other applications received around that time) was invited to comment on Ms Evans' application with it being noted that the cost of funding would have exceeded its previous contribution to the risk-pool. Pharmac told me that its consultation with Counties-Manukau DHB (and the other DHBs) was closely related to the changes that were underway to these budget management processes. Pharmac staff considered that the Myozyme application was a good example with which to demonstrate to DHBs the potential impact of the changes to the budget management of exceptional circumstances funding.

105. Granted all of this, I am still somewhat at a loss to understand what information relevant to the decision that Pharmac had to make, Counties-Manukau DHB could contribute. There were factors, of which the total CPB budget was one, against which Pharmac had to make a decision under its then EC Scheme. It is not easy to see what an individual DHB could contribute to this.

106. Pharmac has told me that it has since determined that the view of a DHB on the financial impact of a proposed funding decision is not a relevant consideration and that no consultation with respect to potential EC Scheme or NPPA Policy funding approvals has occurred since 2011. This appears to be the appropriate position. As Counties-Manukau DHB did not provide any comments to Pharmac on Ms Evans' application, it is not necessary for me to consider the relevance or appropriateness of its contribution to the decision on it.

Pharmac's exceptional circumstances policy

107. As I indicated in describing the role of the Ombudsman, the Ombudsmen are not restricted to examining only the merits of a decision taken by the agency complained of. The Ombudsmen can also examine the law or policy under which that decision was taken and comment on its reasonableness or otherwise. I have done so above in respect of elements of the NPPA Policy that were engaged in making the decisions relating to Ms Evans and I will summarise these in the next section of this opinion.

108. However, while it is open to the Ombudsman to question the law and policy in this way, I think that this jurisdiction must be exercised with discretion. In the case of the law, I conceive that this Ombudsman's jurisdiction is intended to address a situation in which a particular legislative provision operates unfairly or inequitably in the individual circumstance under investigation. Most often this will have occurred because of a legislative oversight that has failed to identify an anomaly that arises in that individual's case. But this is not necessarily always the cause. The individual circumstances may have been appreciated when the legislation was under consideration and a decision to legislate taken regardless. Regardless of the way in which the matter has arisen, an Ombudsman identifying (in his or her view) that such a situation exists is entitled to say that the law in that regard is unreasonable or unjust and to invite it to be re-examined by a suitable authority.

109. But I do not, in essence, see that this is the situation in this case. While there are matters of policy which I intend to invite Pharmac to consider, the major differences between Ms Evans and her supporters, on the one hand, and Pharmac, on the other, are much more fundamental than this.

110. I have said something above about the legal environment, principally the New Zealand Public Health and Disability Act, in which Pharmac operates and the emphasis that this puts on the level of funding provided (ultimately by Ministerial decision) for the tasks it has to perform. It may be that this emphasis results from Pharmac's origins as a subsidiary company wholly engaged in negotiating pharmaceutical prices. In 2001 it became a stand-alone agency with much wider functions, not just to negotiate the best prices for medicines but also with responsibility for deciding what pharmaceuticals deserved public funding in the first place. Despite, or perhaps because of, its new role, its constituent legislation still contains a strong budgetary emphasis.

111. It seems to me that much of the dissatisfaction that Ms Evans' supporters express towards Pharmac stems from a disagreement with this legislative emphasis. This is reflected partly in the desire to incorporate social goals expressly into Pharmac's decision criteria, something that Pharmac has resisted. There is therefore a tension between Pharmac's strong cost-effectiveness approach, which does draw support from the legislation, and the wish of others for less tangible social factors to enter into the equation when exceptional circumstances applications are under consideration. I do not think that as Ombudsman I can resolve these tensions. They are, it seems to me, matters of high policy to be addressed by the Government and Parliament. In this opinion I can

only record this philosophical difference and leave it to others to consider how, if at all, it is to be resolved.

112. On this point Ms Evans emphasises that her disagreement relates not just to legislative emphasis but also to the way in which Pharmac has interpreted the legislation. She says that her approach to how the exceptional circumstances policy should apply also draws support from the legislation and should be acknowledged.

113. I do acknowledge her views on this, but my jurisdiction relates to the reasonableness (or otherwise) of stances that Pharmac takes. In assessing this I am interested in the contrary views expressed on Ms Evans' behalf because these throw light on Pharmac's position and challenge it. But I cannot make a definitive decision on the legal dispute, nor can I say that the opposing arguments are so decisive as to establish that Pharmac's position is contrary to law or unreasonable.

Conclusions

114. As I have described above, section 22 of the Ombudsmen Act authorises the Ombudsman to make findings about an agency's decisions or the law or policy under which such decisions were made, and to make formal (but non-binding) recommendations accordingly. I do not intend to make any findings under section 22 leading to formal recommendations. I do not find matters so clear-cut that one can categorise the actions that have been taken in Ms Evans' case as "*unreasonable*" or "*contrary to law*", nor do I consider that it would be helpful for me to do so. I intend to reiterate in this section some of the views I have expressed earlier when discussing aspects of the decision relating to Ms Evans' application and the policy background to that decision. I will add my view of what I consider should happen next. I am confident that Pharmac as an agency of goodwill will consider my suggestions seriously, indeed Pharmac in its response to my provisional view indicated that it would do so.

115. Not all of my comments do directly affect Ms Evans but it is with her situation that it is critical that we turn first.

116. In this regard I have recorded her clinician's suggestion, when applying under the NPPA Policy, for a limited period of funding for treatment with Myozyme with strict criteria to assess whether to continue on it. As I indicated, I cannot see that Pharmac specifically addressed this suggestion in its response to the application, probably because it had responded to a slightly different suggestion when considering Ms Evans' earlier funding application. I now invite Pharmac to consider any further comments on this suggestion that Ms Evans wishes to make in light of the fuller explanation of Pharmac's position that has been given to me and that I have recorded above.

117. More generally, it seems to me to be desirable for Pharmac more clearly to build an external element into its prioritisation decision-making (possibly through PTAC) since prioritisation has consequences for funding eligibility under the current exceptional circumstances policy that Pharmac operates. I also consider that the NPPA Policy itself

ought to provide that prioritisation will not be a pre-requisite if substantial new information of a clinical or commercial nature becomes available. I suggest too that Pharmac reconsiders its terminology of "prioritisation" to give a clearer indication of the consequences of an assessment of a medicine and its assignment of a priority for funding.

118. Finally, I consider that the decision criteria under the NPPA Policy ought to be clearly differentiated from those under the Pharmaceutical Schedule.

119. These matters are commended for Pharmac's attention.

Addendum – the ability of DHBs to fund medicines

120. In my provisional view, in the course of discussing Pharmac's role in maintaining the Pharmaceutical Schedule and providing for exceptional circumstances funding, I stated that DHBs are not prevented from purchasing medicines that are not on the schedule or otherwise approved by Pharmac (though they were unlikely to do so since they would have to fund them separately).

121. Pharmac, in response, challenged this statement. It pointed to section 23(7) of the New Zealand Public Health and Disability Act which provides:

> *"In performing any of its functions in relation to the supply of pharmaceuticals, a DHB must not act inconsistently with the pharmaceutical schedule."*

122. Pharmac said that with regard to pharmaceutical cancer treatments (PCTs) and community pharmaceuticals, a DHB that provided a medicine not approved under the schedule would be acting contrary to the schedule. (In regard to hospital pharmaceuticals, Pharmac said that this will be the case for them too when it takes over responsibility on 1 July 2013 for determining which medicines can be funded through DHB hospitals.)

123. In response to my query, Pharmac confirmed that as regards PCTs it was referring to clause 5.4.2 of the schedule. Clause 5.4.2 reads as follows:

> *"DHBs must only provide access to Pharmaceuticals for the treatment of cancer that are listed as Pharmaceutical Cancer Treatment in sections A to G of the Schedule, provided that DHBs may provide access to an unlisted pharmaceutical for the treatment of cancer where that unlisted pharmaceutical:*
>
> *a. has Named Patient Pharmaceutical Assessment (NPPA) approval;*
>
> *b. is being used as part of a bona fide clinical trial which has Ethics Committee approval;*
>
> *c. is being used and funded as part of a paediatric oncology service; or*

d. was being used to treat the patient in question prior to 1 July 2005."

124. As regards community pharmaceuticals (which, of course, I am only concerned with insofar as it elucidates the position of PCTs, of which Myozyme is one), Pharmac considered that the same position is achieved implicitly on the basis that the schedule is a complete list of the public subsidies payable for medicines funded in the community and for a DHB to fund a non-approved medicine or one not otherwise authorised, would be inconsistent with the schedule.

125. Pharmac explained the different approach (direct prohibition for PCTs, implicit prohibition for community pharmaceuticals) as probably being due to the minimal opportunity for DHBs to depart from the schedule for community pharmaceuticals. Pharmacies dispensing medicines to patients submit a claim to a central processing centre (operated by the Ministry of Health). For obvious commercial reasons they will not dispense a medicine without charging the patient or ensuring that a subsidy is listed in the schedule or otherwise authorised.

126. PCTs, on the other hand, are generally administered in a hospital setting and there is a greater potential for a hospital pharmacy to purchase an unlisted medicine. At the time Pharmac took on responsibility for the assessment and approval of PCTs in 2002 (when clause 5.4.2 appears to have originated) it did not manage the budget for such treatments, unlike the budget for community pharmaceuticals.

127. NZORD has a different view. In its view a DHB would contravene section 23(7) if it refused to fund a medicine that was on the schedule (in accordance with the eligibility or other criteria prescribed for it). But NZORD does not consider that a DHB would be acting "*inconsistently*" with the schedule if it decided to fund a medicine that is not on the schedule. This is because section 48(b) of the New Zealand Public Health and Disability Act itself recognises that in exceptional circumstances medicines not on the schedule can be funded. If a DHB was said to act inconsistently by funding a medicine not on the schedule it would do that each time it provided a medicine pursuant to the NPPA Policy.

128. NZORD drew my attention to a Ministerial notice of 4 September 2001 (Gazette, 27 September 2001) whereby the Minister of Health authorised an extension of Pharmac's functions to allow it to carry out purchasing on behalf of DHBs. NZORD said that the current Memorandum of Understanding (September 2011) between Pharmac and the DHBs appears to give effect to this extension where it is agreed that non-scheduled pharmaceuticals will be managed via Pharmac processes. DHBs, it said, have thus effectively empowered Pharmac to purchase medicines on their behalf.

129. But NZORD made two points: first, the arrangements between Pharmac and DHBs do not (indeed cannot) legally prevent a DHB ever funding a non-approved medicine and, secondly, if Pharmac is acting as purchasing agent for DHBs it should be taking its purchasing decisions in the light of the statutory functions and responsibilities of DHBs, in addition to its own statutory obligations. NZORD does not consider that Pharmac does this.

130. I do not intend to express an opinion on these latter contentions. I agree that a statutory power cannot be surrendered by a private arrangement with another body. But both Pharmac and DHBs are authorised (ultimately by statute) to take on other functions and to enter into agreements and arrangements with other bodies (including each other). Whether a power can be effectively limited by such an arrangement depends upon a detailed analysis of the provisions that authorise them to be entered into. As to whether Pharmac's enhanced purchasing role imports the DHB's obligations (and whether these are significantly different from Pharmac's), this is a matter beyond the scope of this investigation.

131. But the relationship of section 23(7) to the potential purchase of Myozyme by a DHB is a relevant matter. Myozyme is on the schedule, though not authorised for subsidy. Pharmac says that a DHB is prevented from funding it by section 23(7).

132. I accept that a DHB that provided Myozyme would be acting directly contrary to clause 5.4.2 of the schedule. This, it seems to me, is indisputable. But the real question is whether it is competent to include a provision like clause 5.4.2 on the schedule in the first place.

133. Pharmac's power to maintain and manage a Pharmaceutical Schedule is expressed in section 48(a). (Section 48(b) allowing Pharmac to manage incidental matters arising out of section 48(a), including an exceptional circumstances policy, authorises Pharmac to perform functions separate from the schedule.) Section 23(7) is not a Pharmac power. It is not part of the provision that empowers Pharmac to maintain a schedule. It limits DHBs in their powers by reference to the schedule. But any limitation is predicated on the relevant limitation being authorised to be included on the schedule in the first place.

134. I have seen no statutory authority which allows Pharmac to give directions to DHBs, whether through the schedule or otherwise. DHBs are subject to direction, but by the Minister of Health, not Pharmac (see sections 32, 33, 33A and 33B). I therefore question whether it is competent for Pharmac to address a direction to DHBs in the schedule. Pharmac's function is to maintain a schedule. It is then for DHBs to ensure that they do not act inconsistently with it. Pharmac may legitimately have its own view of what is inconsistent conduct, but I am not convinced that it is authorised to impose that view by a provision in the schedule.

135. Though I do have doubts as to the legal effectiveness of clause 5.4.2 this is not determinative of the question of whether a DHB could, in the face of section 23(7), provide a medicine such as Myozyme out of its own resources and I was wrong in my provisional view to make an unqualified statement that it could. The answer to that question would depend on a consideration similar to that of DHBs funding community pharmaceuticals. In Pharmac's view, as described above, a DHB would be acting inconsistently with the schedule if it funded a community pharmaceutical not approved under the schedule. Presumably the same argument (and NZORD's contrary position) would arise in regard to PCTs. That is a matter on which I do not propose to express an opinion.

Appendix 1. Pharmac's prioritisation process

1. A Pharmac prioritisation meeting for community pharmaceuticals and pharmaceutical cancer treatments occurs three to four times per year and is scheduled by the Manager, Analysis and Assessment, to coincide with the forecast update, and budget bid, as well as meetings of Pharmac's Pharmacology and Therapeutic Advisory Committee (PTAC).

2. Two weeks prior to the meeting the Technology Assessment Group (TAG), which is made up of 6-7 economists/health economists, along with 7 therapeutic group managers (TGMs) compile the list of applications for funding (received from third parties) or proposals for funding (instigated by Pharmac staff) that are to be prioritised. The list is made up of those investment options that are considered to have sufficient information to be able to be prioritised based on Pharmac's decision criteria. Funding options that require further work or follow-up before they can be prioritised are identified separately.

3. A draft prioritisation list is then prepared, which records (by category):

    a. all investment options (in rank order) still 'unfunded' since the last prioritisation meeting;

    b. all new options for investment that are ready to be prioritised;

    c. all new options for investment that are not ready to be prioritised (because further assessment including cost utility analysis needs to occur);

    d. all options for investment that are not ready to be prioritised (because Pharmac is waiting for further clinical information from PTAC or its subcommittees);

    e. all options for investment that are not under active consideration;

    f. all options for investment that have been completed within the last 12 months; and

    g. all options for investment that have been recommended (by clinical advisors) for decline.

4. For each investment option, the draft prioritisation list records:

    a. the medicine and the supplier;

    b. what the medicine treats;

    c. the therapeutic group that the medicine belongs to;

    d. the PTAC priority;

    e. the cost effectiveness (cost per QALY) with likely and possible ranges;

    f. the budget impact to Pharmac – including the impact in the first year of funding and the medium term impact;

g. the budget impact to DHBs (costs in addition to the medicine cost, such as lab testing costs, if any);

h. the cost-effectiveness rank;

i. information relating to Maori and Pacific Island population, including differences in incidence rates conditions and efficacy of medication; and

j. information relating to the health need of patients with the condition the investment relates to.

5. An invitation to the prioritisation meeting is extended to all Pharmac staff. For staff in the following Pharmac roles, attendance is standard:

- Medical Director (or delegate thereof)
- Manager, Analysis & Assessment
- Manger, Access & Optimal Use
- Manager, Corporate & External Relations
- Manager, Schedule & Contracts
- Manager, Funding & Procurement
- Maori health representative
- TGMs
- TAG

6. Prior to the meeting staff attending are provided with the draft prioritisation list, as well as the cost utility analysis matrix and health need tables and graphs. Attendees are required to review and verify the material prior to the meeting.

7. The attendees discuss the investment options and prioritise the proposals for investment by using Pharmac's nine decision criteria (as set out in the Operating Policies and Procedures) to guide the exercise of their collective judgment.