Pharmaceutical Management Agency

Review of Exceptional Circumstances
Consultation on Proposed Changes

January 2011

New Zealand Government

PHARMAC
Pharmaceutical Management Agency

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1. Context and review process

a. Origins and aims

This review of PHARMAC's Exceptional Circumstances (EC) Policy was initiated, in part, in response to the recommendations in the Report of the High-Cost, Highly-Specialised Medicines Review Panel, commissioned by the Minister of Health in 2009.[1]

This review is also in response to the government's *Medicines New Zealand* strategy and its accompanying action plan, *Actioning Medicines New Zealand*[2] and is part of our ongoing work to continue improving our service to New Zealand. Particularly relevant to the EC Policy, *Medicines New Zealand* aims to ensure that 'taking account of and balanced against other health priorities, the medicines system is responsive to individual variation, within a population focus.'[3]

Our aims for this review are to:

- review and clarify the purpose of the provision of funding in exceptional circumstances;
- review and clearly describe what constitutes exceptional circumstances; and
- ensure the operational arrangements for the administration and provision of funding in exceptional circumstances are optimal.

b. Process to date

We sought feedback from a broad range of stakeholders on a discussion document, *Review of Exceptional Circumstances: Seeking Your Views*, which was released in August 2010. The document included key questions about the purpose, criteria, funding and operations of Exceptional Circumstances. We received 76 written submissions from a wide variety of respondents and had two meetings, which included a range of stakeholder groups, during this feedback period. The discussion document, a letter from the PHARMAC Chief Executive inviting responses, and a summary of the submissions we received can be downloaded from our website at www.pharmac.govt.nz/ecreview.

Submitters' responses, and other feedback received on our approach to EC since we assumed responsibility for managing the scheme, have informed our development of the proposals we are consulting on in this document.

c. Seeking your feedback

This document outlines our proposals for changes to EC. We have aimed to describe the proposed approach in sufficient detail to enable you to provide an informed response.

To the extent possible, we have described where our proposals are and are not aligned with submitter feedback in response our discussion document (recognising that stakeholder views are not uniform) and have explained why.

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[1] P McCormack, J Quigley and P Hansen. *Review of Access to High-Cost, Highly-Specialised Medicines in New Zealand*. Report to Minister of Health, Hon Tony Ryall, 31 March 2010. Available at http://www.beehive.govt.nz/release/access-high-cost-medicines-report-received.

[2] *Actioning Medicines New Zealand*, available at http://www.moh.govt.nz/moh.nsf/indexmh/actioning-medicines-nz?Open

[3] *Medicines New Zealand*, available at http://www.moh.govt.nz/moh.nsf/indexmh/medicines-nz

We want to know what you think of our proposals. Questions to help guide your response are included throughout the document and are collated in Appendix 2. Beyond being satisfied that the proposed approach could be implemented subject to feedback in this consultation process, we have not worked out all the implementation details, so have not included these. However, we welcome your comments on any implementation issues associated with our proposals, noting these are subject to change based on feedback. We are also happy to receive any other comments on the proposals that you wish to provide.

This consultation document and an accompanying letter from PHARMAC's Chief Executive inviting your response are also available on our website at www.pharmac.govt.nz/ecreview.

Please pass on this document to anyone who you think may be interested in providing feedback on this topic.

i. Submitting your response

All comments can be submitted via an email, fax or letter by **5pm Friday, 25 March 2011** to:

Bryce Wigodsky
PHARMAC
PO Box 10-254
Wellington 6143

Email: ecreview@pharmac.govt.nz
Telephone: (04) 460 4990
Fax: (04) 460 4995

We also invite interested stakeholders to meet with PHARMAC staff to present their views in response to this consultation. Please contact Bryce Wigodsky by Friday, 18 February 2010 if you would like to meet with us. If a range of stakeholder groups are interested in meeting we may organise larger group meetings.

Please contact Bryce Wigodsky at the above details if you require further information about any other aspects of this review.

Information requested under the Official Information Act

Please note that your response and all correspondence you have with PHARMAC may be the subject of requests under the Official Information Act 1982 (the OIA). *Please indicate if you would like PHARMAC to omit your personal details (name, contact details and any other personally identifying information) from your response, when responding to any request under the OIA*.

If there is any other part of your response or correspondence that you consider could properly be withheld under the OIA, please include comment to this effect along with reasons why you want the information withheld.

d. Next steps

Following receipt of feedback on this consultation document, we will consider all submissions and provide final proposals for any changes to EC to the PHARMAC Board. The Board will be asked to consider making final decisions about any changes to the EC Policy in June 2011. We will be able to advise of the timeframe for implementing any changes following that meeting, and will also release a summary of the submissions received in response to this consultation document.

2. Summary of proposals

The key proposals described in this consultation document are summarised below. References to the relevant section and page number for each key proposal are included to more easily locate further information on the rationale for, and additional details of, the proposals.

- In addition to managing the Pharmaceutical Schedule, PHARMAC is required to provide pharmaceutical funding consideration for patients in exceptional circumstances. We interpret the purpose of this as considering funding in those circumstances in which funding would more appropriately be considered outside the Schedule process.

- We are proposing a new scheme - Named Patient Pharmaceutical Assessment (NPPA) - to provide improved access to pharmaceutical funding consideration for more individuals. The description of the NPPA begins in section 3, page 8.

- We have identified three situations in which we propose a process for individual consideration outside of the Schedule process is appropriate and would improve access to treatments. The proposed NPPA scheme would consist of three pathways, one for each of these situations, under which funding applications for individuals for treatments not assessed for listing on the Schedule at all, or for the relevant indication, would be considered.

- The three proposed pathways of the NPPA scheme are:

    - **Unique Clinical Circumstances (UCC) –** a pathway for individuals whose clinical circumstances are so unique that we would be unlikely to have considered them when deciding whether to list a pharmaceutical on the Schedule. In these situations we would not expect the patient to be part of a group. More details on the purpose of the UCC pathway begins in section 3(a), page 9.

    - **Urgent Assessment** – a pathway for individuals with serious clinical conditions who would experience a significant deterioration in health or lose the opportunity for a significant improvement in quality of life before assessment for listing treatment(s) on the Schedule for the relevant indication is undertaken. This patient may be part of a group of patients with similar conditions. Urgent Assessment would provide wider access as it would take into account factors not considered under the current Community and Cancer Exceptional Circumstances schemes. More details on the purpose of Urgent Assessment are provided in section 3(b), page 9.

    - **Hospital Pharmaceuticals in the Community (HPC**) – a pathway for funding consideration where it would be more affordable for a District Health Board (DHB) to fund the medicine in the community than to pay for the treatment that would otherwise be provided. HPC is similar to the current Hospital Exceptional Circumstances scheme, but we are clarifying that it is the funding of pharmaceuticals outside of the Schedule rather than the circumstances of the individual that is exceptional, and that it must cost less to the DHB as a whole, rather than to the hospital. More details on the purpose of HPC are provided in section 3(c), page 10.

- To achieve the above purposes, each NPPA pathway would consist of a set of prerequisites that would need to be met for an application to be considered and assessed against our decision criteria. Treatments for indications that had already been assessed and declined for

funding at that time, either for Schedule or NPPA funding, would be unlikely to be approved under the NPPA. To assist applying clinicians with their decision making we would provide a list of the medicines and indications we have considered and either approved or declined under the NPPA, as well as those we have assessed for Schedule listing. More details on the prerequisites for each of the NPPA pathways are provided in section 4, page 13.

- Details about the purpose and prerequisites for each NPPA pathway are collated by pathway in Appendix 1.

- The proposals widen the scope of factors we would consider when deciding on funding in exceptional circumstances. The proposals in this document would see a shift in the balance of funding towards funding more pharmaceuticals for individuals prior to a Schedule assessment.

- Consideration of treatments sought under the Urgent Assessment and HPC pathways of NPPA, funded or not, would be more closely tied to consideration for a Schedule listing than is currently the case. Separate from a decision on an NPPA application, we would consider the priority of the treatment being requested for listing on the Schedule. A detailed discussion of this process is provided in section 4(c), page 20.

- Under the proposed Urgent Assessment and HPC pathways, an increased ability to fund pharmaceuticals prior to a Schedule assessment is likely to result in the appearance of some inconsistencies between decisions made under the NPPA scheme, and between NPPA decisions and Schedule listing decisions. More details about this are provided in section 4(c), page 21.

- To reduce applicant confusion and align rationales for assessment pathways we propose removing the distinction between cancer treatments and other community pharmaceuticals. This is a change from the current practice of providing separate funding consideration for these treatments in the Community EC and Cancer EC schemes. More details on the removal of this distinction are provided in section 4(d), page 21.

- Spending on treatments provided in exceptional circumstances is likely to increase, mainly because of wider access available under the proposed Urgent Assessment pathway. Budget management would remain as it is under the current scheme – with funding for NPPA approved treatments drawn from the Community Pharmaceutical Budget. More details on funding and budgeting arrangements are provided in section 5, page 24.

- We propose providing information that would simplify the application process and improve the transparency of decisions on applications. This would include creating a new streamlined application and renewal form, making available a list of treatments and indications we have and have not funded under NPPA, and providing better guidance for clinicians and patients on the NPPA scheme. More details are provided in section 6, page 26.

- The proposed HPC pathway suggests a trial programme exempting DHBs from the requirement to apply for PHARMAC approval prior to dispensing pharmaceuticals that meet the HPC pathway prerequisites and cost less than $500 for a maximum three month course. This would reduce the amount of paperwork required of applying clinicians and help increase the efficiency of the proposed programme. More details about this trial are provided in section 6(a)(ii), page 26.

- PHARMAC would make decisions on NPPA applications with input from a panel of clinicians. Timeframes for response will be determined by the urgency of the circumstances of the named patient the treatment is being sought for. Applicants would be contacted within two working days of our receipt of their application and advised of when a decision can be expected. Further details about decision timeframes are provided in section 6(a)(iii), page 27.

- On occasion, PHARMAC uses the Community EC scheme to provide funding for pharmaceuticals that we have been unable to list in Section B (Community) of the Schedule for reasons not related to exceptionality. Under the proposed NPPA scheme, we would seek to list on the Schedule all pharmaceuticals currently being provided under EC for technical reasons only. This would be in either Section B or a new section of the Schedule. This change would mean that clinicians would no longer need to complete an EC (or NPPA) application for circumstances not related to exceptionality. More details about this are provided in section 7, page 29.

3. The purpose of providing funding in Exceptional Circumstances

One of the key aims of this review of our Exceptional Circumstances (EC) Policy is to clarify the purpose of providing funding in exceptional clinical circumstances.

Section 48(b) of the New Zealand Public Health and Disability Act 2000 (NZPHD Act) establishes, alongside PHARMAC's management of the Pharmaceutical Schedule (described further below) and other functions, PHARMAC's role in managing:

> *incidental matters arising out of [maintaining and managing a pharmaceutical schedule], including in exceptional circumstances providing for subsidies for the supply of pharmaceuticals not on the pharmaceutical schedule.*

The NZPHD Act does not specify what constitutes 'exceptional circumstances' and how the provision is to be implemented.

The Pharmaceutical Schedule is the key mechanism by which PHARMAC achieves its statutory objective. Many submitters to our discussion document expressed the view that 'exceptional circumstances' refers to considering funding in those situations for which the Schedule process does not best cater and we agree with this view. We consider it fair that an alternative process exists to consider funding in such circumstances.

Submitters who responded to the discussion document described a range of exceptional circumstances for which they thought the Schedule process does not sufficiently cater. We considered these suggestions and drew on our own experience of assessing funding applications to identify the situations that, in our view, warrant an additional process to that of Schedule listing.

We consider that there should be a broader range of circumstances in which individuals can apply to have their circumstances considered for pharmaceutical funding. The scheme through which we are proposing such consideration would be provided is 'Named Patient Pharmaceutical Assessment' (NPPA). Under the NPPA Scheme, PHARMAC would consider applications for treatments for individual named patients rather than for listing a pharmaceutical on the Schedule for a population group.

Within the NPPA Scheme, we have identified three pathways by which an individual patient could be considered for funding:

- Unique Clinical Circumstances (UCC)
- Urgent Assessment
- Hospital Pharmaceuticals in the Community (HPC).

Each of these pathways has a set of prerequisites that would have to be met before an application for a named patient would be assessed against our decision criteria and a funding decision made (discussed further in section 4).

The situations that we think warrant consideration under the NPPA scheme, the purpose of such consideration and the reasons why we consider these situations constitute exceptional circumstances are described in more detail in the following subsections (a) – (c). We also explain

in these subsections where the purposes are aligned with those suggested by some stakeholders.

In the final subsection (d) we discuss the reasons that some of the purposes for exceptional circumstances suggested by stakeholders are not reflected in our proposals.

a. Unique Clinical Circumstances

Some patients experience such a unique clinical condition or set of clinical circumstances that we would be unlikely to have considered these when deciding whether to list a pharmaceutical on the Schedule (a process which is designed for population groups). In these situations we would not expect there to be a patient population (e.g. this patient's clinical circumstances would differ from all others who are seeking this particular treatment). In order to meet our statutory obligations we need to assess whether we should fund treatments for individuals in this position. The Schedule process is not the most appropriate process for those patients who differ significantly to the population group(s) seeking the treatment. We therefore consider it would be more appropriate to assess funding for these individuals through the NPPA scheme.

A number of stakeholders who responded to our discussion document commented that EC should provide for conditions that are 'rare' or 'out of the ordinary'. Unique Clinical Circumstances (UCC) provides for truly exceptional cases; although we recognise our proposal is not aligned with the views of those people who wanted to see the number limit on 'rarity' increased from 10. We consider the Urgent Assessment pathway (described in the next section) is the most appropriate avenue for considering the circumstances of those people who are part of a patient population (albeit a small one in some cases).

b. Urgent Assessment

There are some situations in which a patient with a serious clinical condition may experience a significant deterioration in health or lose the opportunity for a significant improvement in quality of life before assessment for listing treatment(s) not listed on the Schedule for the relevant indication is undertaken. This patient may be part of a group of patients with similar conditions. In our view, until the treatment(s) being sought has been subject to a full Schedule assessment for the relevant indication, it is fair to consider funding the treatment for individuals at risk of significant irreversible damage. This proposed change would be significant as it would see the balance shifted in favour of potentially providing funded access to treatments prior to full Schedule assessment.

The circumstances in which we would consider an application under the Urgent Assessment pathway of the NPPA scheme include where the patient's condition may be, but is not necessarily, 'rare' and where provision of the treatment is potentially life saving. These are situations that stakeholders who responded to the discussion document suggested an EC process should provide for. In addition, some medicines likely to be sought by people eligible for consideration under Urgent Assessment could be described as 'high cost' and 'highly specialised'. Improving access to these types of medicines has been a focus for some stakeholders, and this theme was also reflected in submissions.

Under the proposed Urgent Assessment pathway, once a Schedule assessment is completed and the pharmaceutical is either included on PHARMAC's prioritisation list or declined for funding, there would be no justification for considering funding for additional individuals through the NPPA scheme (unless unique clinical circumstances applied). These individuals would be in the same position as others awaiting the outcome of PHARMAC's Schedule funding process. Named patients who had received funded treatments prior to Schedule assessment would continue to

receive funding following assessment, even if the pharmaceutical was not listed on the Schedule for the population, provided they continue to benefit from treatment and meet any conditions of funding at the time of approval. This is discussed further in section 4(c).

c. Hospital Pharmaceuticals in the Community

There are some circumstances in which it would be cheaper for a District Health Board (DHB) to fund an otherwise unfunded pharmaceutical for use in the community for an individual patient under its care, than to pay for the most likely alternative treatment or outcome. DHBs cannot do this without PHARMAC approval as the NZPHD Act requires DHBs to act consistently with the Pharmaceutical Schedule. We consider it fair that a process exists for DHBs to seek PHARMAC approval to be able to supply, and fund from their own budgets, otherwise unfunded treatments.

The Hospital Exceptional Circumstances scheme currently provides this process. The proposed name change to 'Hospital Pharmaceuticals in the Community' (HPC) aims to better reflect the purpose of this proposed pathway and to clarify that under this pathway it is the provision of funding for treatments outside the Schedule that is exceptional, not the clinical circumstances of the individual applicant. This clarification was sought by a number of stakeholders who responded to our discussion document.

We also propose a number of prerequisites for consideration under HPC that better reflect the intent of the pathway than the current Hospital EC criteria. These are described on page 15.

d. Submitter comments on purpose

Philosophical concepts

A range of submitters identified ethical or moral purposes that they considered were relevant to the purpose of funding in exceptional circumstances. These concepts included humanitarian values, ethical imperatives, fairness, patient welfare and equality of access. There was variation between submitters on which of these concepts were more important.

We think of fairness and equity as relating to an equal opportunity for consideration for funding for treatments that improve patient welfare through a process that considers all relevant factors – whether this consideration is for a treatment on the Schedule or for an individual through the proposed Named Patient Pharmaceutical Assessment scheme.

The proposed NPPA scheme would improve opportunities for consideration for pharmaceutical funding for named individuals. Some stakeholders may view the proposed approach as unfair, inequitable or reflecting a lack of consideration of humanitarian values – that groups would not have funded access to treatments they are seeking, whereas individuals may. We do not agree. There is no purpose that we could propose for the exceptional circumstances scheme that would alter the difficult reality that we cannot publicly fund all the treatments that people are seeking. Every health system is limited in how much it can spend on services and it follows that there is a limit on the amount we can spend on treatments, including where there is little evidence of benefit and where greater value may be obtained from using funding elsewhere.

Situations that require an alternative funding mechanism

A sub-set of submitters identified a range of prerequisites that are not specifically provided for in the proposed NPPA pathways described in subsections (a) – (c). These include where treatments are for small groups, people with rare conditions, people requiring expensive treatments, people requiring a treatment for a lifetime and people with unmet health needs.

Submitters expressed the view that the Schedule process does not work for these patients because it penalises applications for treatments for these situations, and also because some of these pharmaceuticals are not currently funded on the Schedule.

The proposed NPPA prerequisites would enable funding consideration for many individuals who would also meet many of the prerequisites suggested by submitters in the preceding paragraph. We gave serious consideration to whether we could target the prerequisites for individual assessment to specifically cover all the situations identified by these submitters. However, we concluded that we would be unable to do this without significantly undermining the Schedule process, chiefly because there is no robust rationale for doing so.

We understand there is a perception that the Schedule assessment process unfairly penalises pharmaceuticals in the situations identified by the sub-group of submitters. However, in our view, the use of the same decision criteria to consider all applications for treatments for population groups under the Schedule listing process ensures the fair and consistent consideration of these applications. We consider there is nothing about the situations described by the sub-group of submitters that warrants a separate process as the Schedule assessment process does not unfairly discriminate against these situations or pharmaceuticals. Our view is aligned with that of the High-Cost, Highly-Specialised Medicines Panel which articulated the need to make decisions in a consistent manner when it recommended that:

> *decisions concerning high-cost, highly-specialised medicines continue to be made in the same way as such decisions for other medicines.*[4]

A single scheme

Many submitters to the discussion document supported the High-Cost, Highly-Specialised Panel's recommendation for a single EC scheme. We have sought to clarify in our proposal that there is one scheme for consideration of treatments for patient groups – the Pharmaceutical Schedule – and another for individuals – Named Patient Pharmaceutical Assessment. Within the NPPA scheme there are three separate situations that we propose to provide for. While one pathway within the NPPA may be desirable, at this stage it would not cover all situations we consider should be provided for. We hope that by more clearly distinguishing between the purposes of the three pathways we will reduce confusion for applying clinicians.

Consultation questions:

1. What are your views on the proposal that the purpose of EC is to provide a scheme for considering funding in those situations where the Schedule process is not the most appropriate?

2. What are your views on the proposal that the Named Patient Pharmaceutical Assessment scheme considers applications for funding for individuals rather than patient populations?

3. What are your views on the proposed purpose of the Unique Clinical Circumstances pathway?

4. What are your views on the proposed purpose of the Urgent Assessment pathway?

[4] P McCormack, J Quigley and P Hansen. *Review of Access to High-Cost, Highly-Specialised Medicines in New Zealand*. Report to Minister of Health, Hon Tony Ryall, 31 March 2010, p 15.

5. What are your views on the proposed purpose of the Hospital Pharmaceuticals in the Community pathway?

6. What, if any, additional situations do you consider the Schedule process does not best cater for that PHARMAC should consider under the Named Patient Pharmaceutical Assessment process?

4. Prerequisites and criteria for named patient applications

This section describes the proposed prerequisites for the three Named Patient Pharmaceutical Assessment pathways outlined in the previous section – Unique Clinical Circumstances (UCC), Urgent Assessment and Hospital Pharmaceuticals in the Community (HPC). A flowchart of the proposed prerequisites is included on page 17.

Meeting the prerequisites would not guarantee that an application for funding under the proposed NPPA scheme would be approved. Applications that satisfy the prerequisites would subsequently be assessed according to PHARMAC's decision criteria before a decision is made about whether the pharmaceutical being applied for would be funded. These criteria are described in sub-section (b) below.

Subsection (c) describes the assessment process for NPPA applications and the implications of the assessment of the same pharmaceutical for Schedule listing. Subsection (d) explains how the proposed approach would remove the distinction between cancer and other treatments that exists with the current Community and Cancer EC schemes, and the rationale for this.

Subsection (e) provides our response to submitters' comments on the criteria they thought were relevant to considering exceptional circumstances applications.

a. Prerequisites for named patient funding consideration

The proposed prerequisite requirements for the UCC, Urgent Assessment and HPC pathways are detailed in subsections (i)-(iv) below. Applicants would need to satisfy these prerequisites in order for their application to be considered for funding under our decision criteria and a decision made.

PHARMAC would be unlikely to approve any treatments for any patient under the NPPA scheme if we had already assessed for Schedule listing the pharmaceutical being sought for their equivalent clinical circumstances. We would maintain a list of such medicines and indications on our website which clinicians could check when deciding whether to apply for NPPA funding.

We would continue to exercise our discretion to determine the most appropriate pathway for an application under the NPPA scheme based on the information that is provided. This means that if we received an application that did not meet the prerequisites for the Unique Clinical Circumstances pathway (for example, because we were aware of other patients with the same clinical circumstances) we would consider whether the application met the prerequisites for the Urgent Assessment or HPC pathways and make a decision on it accordingly. We would naturally advise the applicant of this at the time and seek more information to support the application if this was required.

In addition to the prerequisites proposed here, stakeholders are always welcome to seek PHARMAC's agreement to fund pharmaceuticals for named patients that are less expensive than the treatments listed on the Schedule. PHARMAC would need to consider, alongside other factors, whether the pharmaceutical being sought was actually cheaper than the funded alternatives. Confidential rebates on some products mean that the Schedule price listed for some pharmaceuticals is higher than the price paid.

i. Unique Clinical Circumstances

As discussed in section 3(a), some individuals experience clinical circumstances which are so unusual or unanticipated that PHARMAC is very unlikely to have considered these through the Schedule listing process and we would not anticipate seeing them in more than one patient. We consider it appropriate to provide a process for considering funding for such circumstances – which we refer to as Unique Clinical Circumstances.

The proposed prerequisites for the UCC pathway of NPPA are that the patient:

1. has tried and failed all alternative funded treatments (or alternative treatments have been contraindicated), or has experienced such serious side effects with all other relevant funded treatments that treatment has been ceased; AND

2. is experiencing an indication or set of clinical circumstances that are unique to them and make their case different from all other patients with the same condition.

Consultation question:

7. What are your views on the proposed prerequisites for the Unique Clinical Circumstances pathway?

ii. Urgent Assessment

As outlined in section 3(b), assessing applications under the Urgent Assessment pathway of the NPPA scheme would enable PHARMAC to consider funding a treatment that has not been assessed for Schedule listing for the relevant indication for a named patient with severe clinical circumstances who would, prior to the pharmaceutical being assessed for the Schedule, either:

- experience a significant deterioration in their health if they did not receive the pharmaceutical that is not currently funded on the Schedule, or
- would lose the opportunity for a significant improvement in quality of life.

The capacity for significant benefit, or the avoidance of significant harm, is key to this pathway. There must be a real expectation that there is a chance of significant recovery in health or maintenance of health where significant deterioration is otherwise inevitable.

We think it is fair to consider funding treatments for individuals in these circumstances until the treatment has been assessed for listing on the Schedule. At the point at which a Schedule listing is completed there would be no justification to assesses applications for named patients as they would be in the same position as others seeking treatments on PHARMAC's prioritisation list.

The proposed prerequisites for the Urgent Assessment pathway of NPPA are that:

1. The patient has tried and failed all alternative funded treatments (or alternative treatments have been contraindicated) OR has experienced such serious side effects with all other relevant funded treatments that treatment has been ceased; AND

2. The indication or set of clinical circumstances may affect more than one patient (either currently or over time); AND

3. The patient has a serious clinical condition and not receiving the treatment prior to Schedule assessment would lead to either:
    - a significant deterioration in a serious clinical condition, OR
    - the patient would miss the opportunity for significant improvement in quality of life.

Consultation question:

8. What are your views on the proposed prerequisites for the Urgent Assessment pathway?

iii. Hospital Pharmaceuticals in the Community

As outlined in section 3(c), PHARMAC recognises there are situations in which funding a community treatment not listed on the Schedule for a short time will be cheaper for a DHB than the alternative intervention or outcome. The HPC pathway provides a process for DHBs to seek approval to fund treatment in these circumstances.

The proposed prerequisites for assessment for funding under HPC are that:

1. the patient has tried and failed all alternative funded treatments (or alternative treatments have been contraindicated) or has experienced such serious side effects with all other relevant funded treatments that treatment has been ceased; AND

2. the application is for a DHB hospital to fund a treatment for use in the community for a patient under the care of a DHB hospital clinician (in-patient or out-patient); AND

3. the treatment is not a cancer treatment; AND

4. the treatment costs less for the DHB than the most likely alternative intervention or outcome; AND

5. the length of treatment is less than three months (noting that PHARMAC may approve treatments for longer than three months in compelling cases).

We propose trialling giving DHBs discretion to dispense, without prior PHARMAC approval, treatments that cost $500 or under and meet the prerequisites listed above.

Cancer treatments

As PHARMAC manages both the community and hospital funding of pharmaceutical cancer treatments, we consider it would be inappropriate for DHB hospitals to fund cancer treatments from their own budgets. Pharmaceutical cancer treatments would be eligible to be considered under the UCC and Urgent Assessment pathways, subject to meeting the relevant prerequisites.

Lower cost to the DHB than the alternative intervention or outcome

When considering the most likely alternative treatment, PHARMAC will generally not consider admitting the patient solely for the purpose of providing the treatment to be adequate to meet the prerequisite requirements for HPC.

Time limits on treatment length

The proposed criterion limiting the length of treatment to three months clarifies that funding treatments through the HPC pathway is not intended to be an alternative to a Schedule listing. Previously, where long-term treatments have been funded for a large number of patients through Hospital EC, PHARMAC has had difficulty when subsequently negotiating a Schedule listing because an alternative market had been created.

While we propose to limit the length of HPC funded treatments to three months, PHARMAC would be able to use its discretion to approve funding for longer time periods where this is clinically appropriate. We would expect to exercise this discretion only where a compelling case is made as to why a community pharmaceutical treatment that is not listed on the Pharmaceutical Schedule should be provided to a patient for a longer time period.

Pharmaceuticals under $500

The lower the cost of the pharmaceutical treatment the more likely it will be cheaper for the DHB compared with the alternative intervention or outcome. Recognising this, and that the application process is an administrative burden for applicants, we consider it desirable to trial setting a cost limit under which DHB hospital clinicians would be able to dispense, without PHARMAC approval, community treatments that are not listed on the Schedule. The proposed limit for the trial is $500 for the treatment course, and the HPC prerequisites - including that treatment course is not longer than three months – would need to be met. Clinicians wishing to seek approval for funding a treatment for a longer duration would need to make an HPC application.

DHB hospitals wanting to participate in the trial would need to be able to provide PHARMAC with patient level data on those pharmaceuticals funded through HPC under $500 to enable PHARMAC to monitor prescribing. Such monitoring would be essential to ensure we could take action if it appeared that a market was being developed such that there was a risk that negotiation for listing on the Pharmaceutical Schedule could be undermined. If PHARMAC considered there was a risk with the funding of community treatments under HPC (whether under the limit or not), DHB hospitals would not be permitted to dispense these and we would decline all HPC applications for these products. Further details on the arrangements for the 'under $500' trial are described in more detail in section 6(a)(ii).

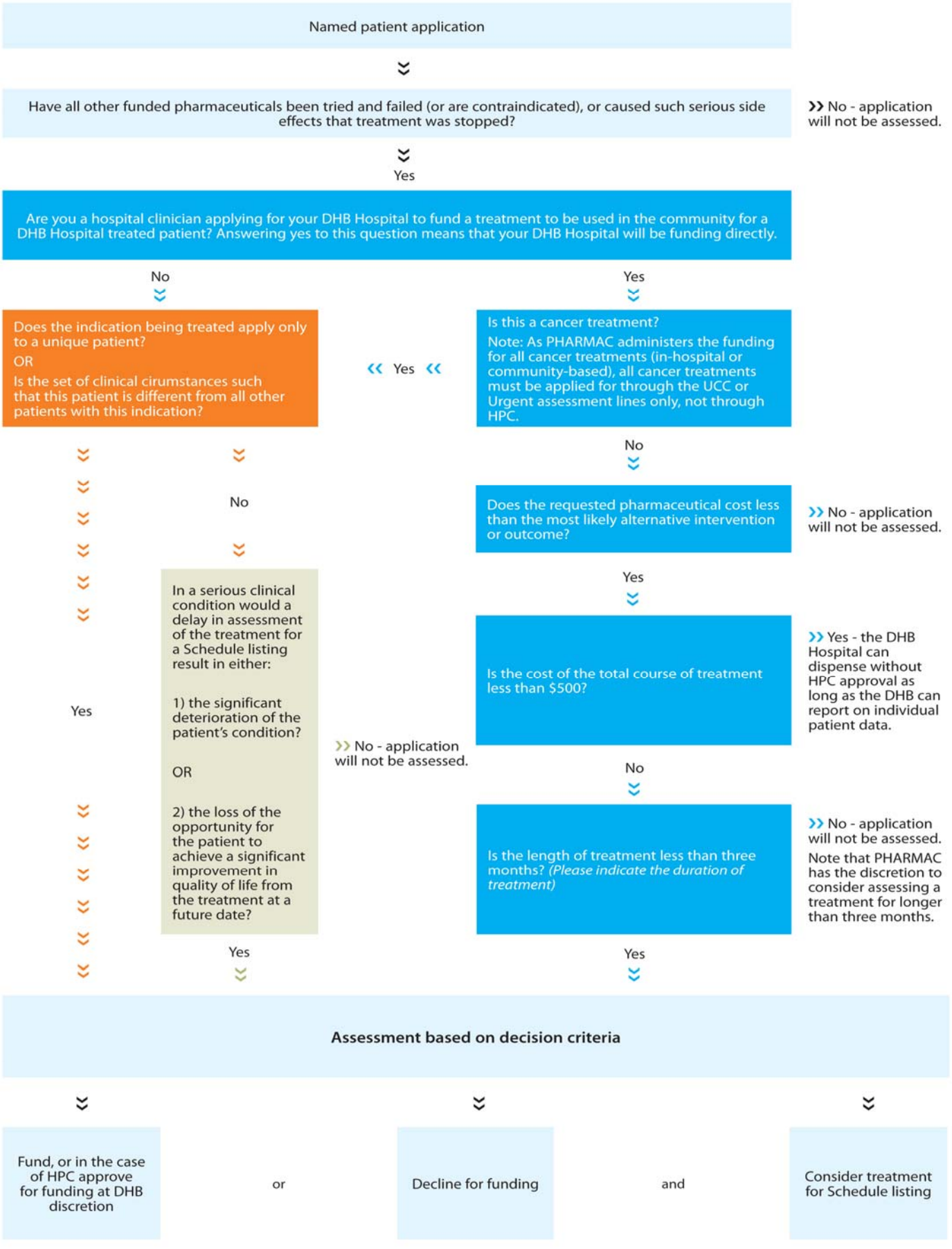
Consultation question:

9. What are your views on the proposed prerequisites for the Hospital Pharmaceuticals in the Community pathway?

iv. Summary prerequisites flow chart

The flowchart below lists the proposed prerequisites for the Named Patient Pharmaceutical Assessment pathways.

Named Patient Pharmaceutical Assessment Process



b. Named patient pharmaceutical application assessment criteria

Applications which satisfy the prerequisites described above would be assessed according to PHARMAC's decision criteria before a decision was made about whether the application would be approved or declined.

The criteria, and some of the considerations that may be relevant to these, are listed below. As explained in the table, some of the criteria that are appropriate for us to consider when we are determining whether to fund a treatment for a population group are not relevant to decisions on individual applications.

Considerations that are in **bold** are those that some submitters who responded to our discussion document thought should be taken into account in decisions.

Criteria	Considerations that may be relevant
1. The health needs of all eligible people.	The health needs of the named patient (including **consideration of severity of condition and urgent need for treatment)** and the **unmet need** that would be met by the treatment that is being sought.
2. Particular health needs of Maori and Pacific peoples.	The health needs of the individual and the likely population is a consideration.
3. The availability and suitability of existing medicines.	Whether there are other treatments (pharmaceutical or otherwise) that could meet the health needs identified under criterion 1. **Why these existing interventions are not available to/suitable for the named patient.**
4. The clinical benefits and risks.	**What the additional health benefit is that this treatment would provide over the funded alternatives (if any) suitable for use by the named patient. This includes an assessment of available evidence relevant to the indication.** What the side-effect profile of the treatment is compared to the funded alternatives (if any) suitable for use by the named patient.
5. Cost effectiveness of meeting health needs through pharmaceuticals rather than other services.	**What the cost-effectiveness of funding the pharmaceutical would be. This includes considering the cost-effectiveness of a treatment for a target group where there is evidence that demonstrates a different treatment effect to the broader patient group.** How the cost-effectiveness of this treatment compares with treatments that are funded on the Schedule.
6. The budgetary impact (in terms of the pharmaceutical budget and the Government's overall health budget) of any changes to the Pharmaceutical Schedule.	The budgetary impact for NPPA is considered under criteria 9.
7. The direct cost to health users.	How funding this treatment would affect the direct costs to patients. This is not about whether the named patient could afford to privately fund the treatment, but whether this would increase or decrease co-payments, number of doctor visits or other direct costs borne by them.

	This excludes indirect costs, such as transport costs, changes in income, and social costs including childcare costs etc.
8. Government's priorities for health funding.	Government priorities are routinely considered for Schedule listing applications and may be considered for relevance here.
9. Such other criteria as PHARMAC sees fit.	Any other considerations that PHARMAC considers it appropriate to take into account. One such consideration we think would be relevant to all NPPAP applications is: The budgetary impact of any NPPA funding decision. Related considerations include: • **The impact of the cost of the treatment on the Named Patient Pharmaceutical funding provision and the Community Pharmaceuticals Budget, of which this is part.** • The likely number of patients in the same circumstances seeking this treatment. • The relative priority of this treatment on our list of products to assess for Schedule listing. • The speed with which we would need to undertake a Schedule assessment for this product, and the resources available to do this. • The implications that funding the treatment for the named patient, and others in the same circumstance, may raise for trying to secure a Schedule listing.

We propose that it would not be relevant to our assessment of a NPPA application whether the patient has started a pharmaceutical treatment overseas or has self-funded it. We also propose that where self-funding has occurred, any resulting information on the effectiveness of the treatment would not be considered in any PHARMAC decision on a Named Patient Pharmaceutical Application. This is not a new policy, but a continuation of an approach that recognises that those who have the ability to self-fund treatment, or to travel overseas to initiate a treatment, should not benefit more from the publicly-funded health system than those who do not have the means to do this.

Consultation question:

10. What are your views on the proposal to assess Named Patient Pharmaceutical Applications against PHARMAC's decision criteria?

c. Named patient and Schedule listing assessments

The proposed Urgent Assessment and HPC pathways would see the balance of funding shifted in favour of PHARMAC providing funded access to some treatments prior to full Schedule assessment. Currently, patients have to wait for treatments to be listed on the Schedule to get funded access to them unless they fall under the criteria for the current EC scheme which are significantly narrower than the prerequisites for the Urgent Assessment pathway.

Assessment for the Schedule

PHARMAC would, separate from deciding on an application for a pharmaceutical for a named patient, consider the priority with which we would undertake a Schedule assessment for that pharmaceutical if one was not already underway. Undertaking a Schedule assessment would ensure that PHARMAC would also consider the provision of treatments being sought by named patients for listing on the Schedule for the population.

PHARMAC would not necessarily list a pharmaceutical being funded for a named patient on the Schedule within any particular timeframe, or at all. The information relevant to PHARMAC's decision criteria (including the available budget, clinical evidence and competing priorities for funding) would differ at different points in time.

When undertaking a Schedule assessment PHARMAC would draw on a much more comprehensive set of information than would be considered for a NPPA application to determine whether the pharmaceutical is one we would wish to list on the Schedule and its relative priority compared with other funding options. This information may reveal that the pharmaceutical is a poor option compared with other treatments we are considering for funding. Alternatively, Schedule assessment may indicate that the pharmaceutical is of high value. As with all Schedule funding decisions, the speed of listing products on the Schedule that are being funded for named patients would depend on the relative priority compared with other options, and the available budget for new investments.

At the point at which a pharmaceutical has been assessed for the Schedule, we would not approve any further applications for named patients (even if we intended to list the pharmaceutical at some point on the Schedule). The rationale for funding for named patients is that it is fair to consider their circumstances for funding prior to undertaking a Schedule assessment. However, once this assessment has been completed, patients seeking the pharmaceutical would be in the same situation as all others awaiting Schedule listing of treatments that we have assessed. There would be no rationale for treating people in this situation any differently. We would, however, continue funding patients who had been approved for funding through the NPPA scheme prior to the Schedule assessment being undertaken.

Because pharmaceuticals funded under the UCC pathway would be for one-off situations, Schedule listing decisions would be unlikely to have any implications for subsequent applications as they would for the Urgent Assessment and HPC pathways.

Named patient assessment

A decision by PHARMAC to fund a pharmaceutical for a named patient would not signal that we would necessarily approve for funding a subsequent application for the same pharmaceutical for a named patient with the same clinical circumstances who was applying at a different point in time. As for Schedule listing decisions discussed above, our decisions on NPPA applications made at different points in time for people in the same clinical circumstances may differ due to the different information, relevant to the decision criteria, which may be available.

When assessing the first application for a treatment for a named individual we may not have sufficient information to be able to determine the value of the treatment (including the magnitude of benefits for the individual). Given the likely uncertainty of treatment benefit, we would need to carefully consider the risks when deciding whether or not to fund for the named patient prior to undertaking a Schedule assessment. These include the risk to the budget of funding an unprioritised treatment and the risk of creating a market which may undermine effective future

negotiation for Schedule listing. We would need to estimate a number of factors (including likely numbers of people seeking the treatment through the NPPA scheme) to help us make this decision. If, over time, more information became available that revealed our assumptions were incorrect, our decisions on subsequent NPPA applications for people in the same clinical circumstances may differ. This could mean that we would approve funding for a pharmaceutical for one or some named patients, and subsequently decline applications for the same pharmaceutical for people in the same clinical situations because new information has come to light. We would maintain, on our website, a list of treatments and indications approved and declined under NPPA as a guide for clinicians considering submitting applications.

Apparent inconsistencies in decisions

We are aware that under the scenarios described above our decision-making may appear to be inequitable or inconsistent. This potential for apparent inconsistencies in decisions already exists within the Schedule decision process where the amount of funding, priorities and available evidence may vary from year to year. That the decisions would be made at different points in time taking into account different information would not change the fact that people with the same clinical circumstances may end up with different funding results. However, shifting the balance to more routinely consider funding pharmaceuticals for named patients prior to full assessment of these pharmaceuticals for Schedule listing relies on us being able to make decisions that may differ over time. We recognise that it is important to gather stakeholders' views on the acceptability of such an approach and have included specific consultation questions on this below. If stakeholders view the potential scenarios described above as unacceptable, we would take a more conservative approach to approval of NPPA applications to manage the risk of significant budget impact.

Consultation questions:

11. What are your views on the proposal that an NPPA application (other than within the UCC pathway) triggers PHARMAC's consideration of listing the treatment being requested on the Pharmaceutical Schedule?

12. Do you favour wider access with the appearance of inconsistent decisions that this may bring, or a more restrictive but consistent approach?

d. Combining cancer and community treatment consideration

The proposed prerequisites and decision criteria for the UCC and Urgent Assessment pathways would apply equally to NPPA funding applications for all community pharmaceuticals and hospital cancer pharmaceuticals (section 4(a)(iii) explains why cancer treatments would not be considered under the HPC pathway). This would be a change from the current approach, in which different criteria are applied to cancer treatments (through the Cancer EC scheme) compared to other treatments (through the Community EC scheme). We consider it appropriate to align these two schemes because:

- The same standards would be applied to all treatments PHARMAC is responsible for, regardless of whether the treatment is for cancer or another indication - a common theme discussed by a number of submitters to the discussion document. Because PHARMAC does not currently manage the budget for in-hospital non-cancer treatments, consideration of these applications will need to continue to be assessed differently in the medium-term (under HPC).

- Alignment would move towards reducing confusion for applicants about which scheme to apply under. The same treatment may sometimes be used for either cancer or other indications, and currently applicants must determine which scheme is the most appropriate to apply under.

- The underlying rationale for the current Cancer EC and Community EC schemes is the same – the need for a scheme enabling funding consideration for treatments for individuals in specific circumstances.

Paediatric cancer treatments would continue under the current notification system, where funding is granted without an application being required. PHARMAC considers it pragmatic to continue to permit the paediatric exemption at the current time. This will likely be reviewed again as PHARMAC considers wider in-hospital processes.

Consultation question:

13. What are your views on removing the distinction between cancer and community treatments under the proposed NPPA scheme?

e. Submitter comments on criteria

Submitter comments on the criteria for any new scheme naturally overlapped with discussions of the purpose of the scheme. Section 3(d) discusses our response to some of the key points raised by submitters.

Rarity

A large majority of respondents to our discussion document supported the use of a rarity criterion to define exceptional, but there was wide variation in views as to the number that constituted rarity. A number of respondents considered that the figure of ten for the current scheme was too low, with two suggesting that the number be increased to 100. Some individuals considered that the criterion needed to remain tight to ensure that applications were truly exceptional. The main concern was that all applications have somewhere to be considered.

As discussed in section 3(d), the fact that a pharmaceutical treats a rare condition does not, in itself, disadvantage it in the Schedule assessment process. PHARMAC's assessment determines the value offered by a medicine relative to its cost, regardless of the size of the population group that stands to benefit. If the population in which the medicine is effective is small (either because the medicine is targeted to a subset of a disease group or because the disease itself is rare) the value and cost is assessed for this patient group (not the broader population).

In our view, standard treatments for standard conditions, regardless of the number of people they affect, should be considered through the Schedule process. The UCC pathway is designed to cater for those situations which we would never have considered for a Schedule listing.

In addition, named patients seeking funded access to pharmaceuticals for rare conditions (as well as those whose conditions are not rare) would be able to apply for consideration under the NPPA if their circumstances met the prerequisites for the Urgent Assessment pathway.

Other criteria

We highlighted in section 4(b) those criteria that respondents to our discussion document thought we should consider that may be relevant under our decision criteria. These include intolerance and ineffectiveness of treatments, evidence, cost-effectiveness, disease state and consequences of non-treatment.

With regard to evidence, some submitters commented that a new EC scheme should provide funding for a trial of a new treatment or for a rare condition where supporting data is limited. PHARMAC already provides trials for some treatments where treatment response criteria need to be met for continued funding for individual patients. Under the NPPA scheme, PHARMAC would consider all applications for pharmaceutical funding and would fund those that met the prerequisites and were assessed favourably against the decision criteria. Treatments or conditions with limited data may qualify under these criteria. However, this is ultimately a judgement call, and very expensive treatments with little evidence of effectiveness may be less likely to be funded.

Some submitters considered that some non-clinical factors (such as age, contribution as a tax-payer and religion) and benefits to the wider society, not just to the health system, should be considered in decision-making to recognise that some pharmaceutical funding decisions will have a financial impact on other government sectors. Proposals to factor this into decisions raise a number of theoretical and practical issues including:

- ethical and legal considerations – e.g. should the needs of paid workers be valued more highly than children/elderly people? To do so may well be considered discriminatory under the Human Rights Act;
- differences in assumptions used by other government agencies resulting in inconsistent analyses; and
- the difficulty and cost of PHARMAC accurately estimating the impacts of potential decisions to areas outside the health sector.

In addition, there is no real-time ability to transfer funds and resources between government agencies. If PHARMAC was to start taking these factors into account without any such transfer of funds, the health gains achieved from pharmaceutical funding would be significantly less than they are currently.

Pharmaceutical funding is not the only area of public management in which a decision made in one area has an impact on costs to government in another area. For example, decisions about improvements to New Zealand's roads would have an impact on the health sector through reducing, or not, the number of road accidents on identified 'trouble spots'. The role of government is to take into account the interdependencies of individual portfolios when allocating funding to competing areas of public management, so that continual transfers of funding between public agencies, based on the potential effects of their decisions, is not required.

5. Funding

a. Named Patient Pharmaceutical Assessment funding provision

We propose that the funding for treatments approved for named patients under the UCC and Urgent Assessment pathways would be allocated in the same manner as the funding currently is for Community EC. The NPPA funding provision for approved NPPA applications would be top-sliced from the Community Pharmaceutical Budget (CPB). If a treatment that is funded for named patients was subsequently listed on the Schedule, funding for the treatment would then come from the Pharmaceutical Schedule portion of the CPB rather than the top-sliced NPPA provision. We propose that DHB Hospitals would continue to fund treatments approved by PHARMAC under the HPC pathway.

The NPPA funding provision would be agreed between PHARMAC and DHBs and would be risk-pooled to protect those DHBs with patients who require very expensive treatments. At this time, we are unable to estimate the volume or cost of NPPA applications that would be likely to be approved under the proposed new approach. This means we cannot pin-point an appropriate figure for the NPPA funding provision. However, we estimate that, initially, we would need to increase the funding provision to ensure resources are available to fund approved NPPA applications and would need to agree such an increase with DHBs. We anticipate this increase would be able to be absorbed within recent increases to the CPB.

Should it become apparent that funding for NPPA treatments would exceed the agreed provision (including any increase) we would seek agreement from DHBs to increase the funding provision to continue funding all NPPA applications that meet the prerequisites and criteria. Because the NPPA funding provision is met from within the CPB, any increase in NPPA expenditure would reduce the level of CPB funding available for other pharmaceutical investments. Additionally, funds not committed for NPPA would be able to be used to fund community pharmaceuticals listed on the Schedule, as is presently done.

Central management of funding for cancer treatments approved for named patients (which would be funded from the NPPA funding provision) would reduce the inconsistency that currently occurs when DHBs make different decisions about which cancer treatments they will fund outside the Schedule. Reducing inequity in funding was a key theme expressed by submitters in responses to our discussion document.

b. Submitter comments on funding

Some submitters to the first feedback phase of this review commented that the budget for pharmaceutical exceptional circumstances funding should be increased and made more flexible. As discussed above, we consider we would need to increase the funding provision for NPPA applications. We also always have the ability to seek DHB agreement to increase the NPPA funding provision. This occurred in 2003 when the EC funding provision was increased from $2.5 million to $3 million per annum.

Some submitters suggested that setting the notional budget for any new approach to exceptional circumstances should occur separately from setting the CPB. Other submitters thought that separate pools of funding for EC and for high cost and/or highly specialised medicines were necessary. These submitters felt this approach would address the gaps they consider exist in the current EC system.

PHARMAC shares the view of the High-Cost, Highly-Specialised Medicines Panel, which recommended against the establishment of a separate funding pool for high-cost, highly-specialised medicines. It is important to recognise that more funding for pharmaceuticals approved under NPPA will mean less money is available for treatments funded through the Schedule. In addition, just as there is no 'right amount' for the CPB, it would also be impossible to determine a 'right amount' of funding for NPPA treatments.

While the focus on the size of the NPPA funding provision is understandable, it is not a measure of the success of an exceptional circumstances scheme. Our aim would be to, over time, list on the Schedule those treatments initially funded under the NPPA which compare favourably with other treatment options. Funding for these treatments would no longer need to be provided from the NPPA funding provision. The proposal to continue to treat NPPA expenditure as expenditure within the CPB, as is presently the case, is considered an integral part of PHARMAC's overall budget management.

Part-funding

Two submitters commented on the possibility to allow for part funding of EC treatments by PHARMAC and patients, to free up more funding for wider access to EC. One of these also noted that allowing part-payments by a patient may discriminate against patients who are unable to pay for even part of a treatment. PHARMAC agrees that part-funding can create inequity, as well as significantly reduce the value for money that we can obtain from pharmaceutical funding for all patients.

Reducing inequity

Central management of funding for cancer treatments funded under NPPA will reduce the current inequity in access that arises from DHBs taking different approach to funding treatments under Cancer EC.

PHARMAC will not be able to fully address concerns about inequity in the provision of funding for the current Hospital EC scheme, which would not be addressed by the proposed Hospital Pharmaceuticals in the Community until such time as it holds the budget for these treatments. This is an issue we will be keeping in mind as we consider processes around our new activities in the hospital environment.

Consultation question:

14. What are your views on the proposed approach to funding for treatments approved under the NPPA scheme?

6. Operational arrangements

a. Named Patient Pharmaceutical Assessment

i. Making applications

PHARMAC proposes creating a new NPPA application form, which would be made available on the PHARMAC website. This would clearly describe the information applicants would need to include to have their application considered under the NPPA scheme. PHARMAC would also provide updated and expanded information sheets explaining the scheme, both for clinician applicants and for patients for whom applications are being made.

We would investigate the feasibility of applicants completing and submitting NPPA application forms electronically to reduce the administrative burden for applicants.

PHARMAC would maintain a list of the pharmaceuticals and indications it had assessed for listing on the Schedule and those it had approved and declined under the NPPA scheme. Clinicians could check this list to help them to decide whether to make an application.

The proposed changes listed above would work towards simplifying the application process and increasing accessibility and transparency of information – all of which were themes raised in submissions.

ii. Exemption from application for 'low cost' hospital pharmaceuticals

As discussed in section 4(a)(iii), we propose trialling the use of a cost limit under which a clinician would not need to make a funding application if they wanted to dispense a pharmaceutical to a patient in line with the HPC prerequisite requirements. Such an approach was sought by a number of submitters who responded to our first document.

For DHBs to take advantage of this trial they would need to be able to have a system in place whereby clinicians could rapidly identify whether a full course of treatment for the pharmaceutical they wished to provide would fall under the cost limit of $500 for a treatment course of not longer than three months (and whether they needed to make an application or not).

DHBs would also need to be able to report to PHARMAC monthly on what is being funded under this threshold. In particular, they would need to provide non-identifiable patient-level data on the details of the pharmaceuticals being funded and their cost. DHB hospitals that are unable to report this information to PHARMAC would continue to be required to apply to PHARMAC for approval to dispense community pharmaceuticals under $500. Ideally, DHBs would also be able to distinguish in their reporting between pharmaceuticals funded under Discretionary Supply, HPC and under the cost limit of $500.

PHARMAC would use the information provided by DHBs to assess the risks of funding outside the Schedule process. Specifically, if it appeared that a market was being created for a pharmaceutical that may undermine future negotiations for Schedule listing, PHARMAC would advise all DHBs that the pharmaceutical could no longer be dispensed under HPC (regardless of the cost of the treatment). The information would also be used to assess whether treatments dispensed in this category should be considered for Schedule listing.

In addition, if data revealed that a DHB was dispensing community treatments outside the HPC prerequisite requirements, PHARMAC may require the DHB to apply for approval for all HPC treatments, irrespective of whether the cost is under the $500 threshold or not.

The Schedule would be updated to include a rule enabling DHBs to provide funded access while still acting consistently with the Schedule.

iii. Considering applications

Under the proposed approach, PHARMAC would make decisions on named patient applications, on advice from the NPPA Panel (which would have the same composition as the current EC Panel). PHARMAC has recently appointed two haematologists as advisors to the EC Panel and is seeking oncology representation to provide additional input into Cancer EC applications.

It was clear from submitters' views in response to our discussion document that the timeframes within which we would respond to applications would be critical. Currently, the timeframe for a decision on an application is determined by the EC scheme under which the application is submitted. Under the new approach, PHARMAC would assess first those applications that, due to the patient's circumstances, require the quickest decision irrespective of the NPPA pathway that has been applied under. All applicants would be contacted within two working days to advise them of when they could expect a decision on their application, and some applications would be decided within this time period. The majority of NPPA applications will have a decision within one month with the exception of those requiring a Board decision. The applying clinician would need to include in their application some detail on how urgent they consider the application to be.

iv. Communicating decisions to applicants

All decisions for individual patients under this proposal would be communicated to applicants by their preferred method, including email. Recognising the importance of transparency of the reasons that an application was funded or declined (a key theme in submissions), PHARMAC would provide an explanation for the decision to the applicant.

Under NPPA, applicants would continue to be encouraged to present additional information for consideration by PHARMAC and the NPPA Panel where appropriate.

v. Renewals

Some submitters commented that renewals (or re-applications) should not be required where a patient's clinical circumstances are unlikely to change. PHARMAC would have the discretion to set the timeframe within which UCC and Urgent Assessment renewal applications will be considered – and could provide longer timeframes where this is warranted.

A new form would be developed for NPPA renewal applications. Renewal applications would need to continue providing a full clinical update on the patient's progress in order to justify continued funding. PHARMAC would examine the original application and the newly submitted renewal information, seeking advice from the Panel where appropriate.

Renewal applications for the proposed HPC pathway of NPPA would not normally be accepted as the proposed HPC funding prerequisites explicitly state that treatment would be no longer than three months. PHARMAC would remain able to exercise its discretion to approve a renewal if appropriate.

vi. Review process

Under NPPA, all applicants would be able to request a review of their application if they were not satisfied with the decision. This review would be undertaken by PHARMAC staff who would be able to seek any external advice they considered necessary, including from an external clinical panel.

vii. Information about decision outcome

PHARMAC proposes to begin providing a summary of all applications under NPPA on our website. Subject to privacy considerations, information in this summary would include the medication requested, the indication it was requested for and PHARMAC's decision.

viii. Transition arrangements

Should some or all of the changes proposed in this document be implemented, careful attention will need to be given to transitioning from EC to NPPA.

We are aware there are some patients currently receiving EC funding for a treatment who would not qualify for funding under these proposals. PHARMAC would continue funding all patients receiving EC treatment who continue to qualify for funding under the current EC criteria.

b. Submitter comments on operational arrangements

Our proposals for operational arrangements reflect a number of improvements sought by submitters in their responses to our information seeking phase.

A number of submitters commented on the assessment process for applications. The suggestion was made that each application be examined by a relevant specialist. However, with more than 25 specialist fields, PHARMAC does not consider it practical to do so whilst still ensuring parity between applications in different fields and also ensuring that rapid responses are made to applicants.

In advance of this EC Review, and as part of our on-going improvements, we have recently added two haematology advisors and are seeking oncology advisors to the EC Panel. These advisors will be consulted on current Cancer EC applications. We propose using these advisors for assessment of any NPPA applications for cancer indications.

The NPPA Panel and PHARMAC would remain free to seek any additional advice as they see fit. Applicants may provide additional information and request a review (section 7(a)(vi)) should they consider that an incorrect decision has been made.

Consultation questions:

15. What are your views on the proposed operational arrangements for the NPPA scheme?

16. What information do DHB hospitals routinely collect and how would this satisfy the information needs we have identified as necessary from DHB hospitals wishing to participate in the trial described in section 6(a)(ii).

7. Funding treatments for populations on the Schedule

PHARMAC has previously used the Exceptional Circumstances mechanism (including forms and approval numbers for claiming) to provide funded access to pharmaceuticals in circumstances that would not be defined as exceptional under the purpose statements described in section 3.

Previously, PHARMAC has not been able to achieve a Section B (Community) Schedule listing for some pharmaceuticals that we wished to provide for a patient group rather than an individual. In most cases this was due to historical rules about what could and could not be listed on the Schedule. For example, we previously did not list unregistered pharmaceuticals or pharmaceuticals for unregistered indications and we also preferred not to list medicines for which we had no supplier contract. In these situations, the Community EC mechanism has been used to provide funded access to pharmaceuticals for individuals. PHARMAC's use of the EC mechanism to implement funding decisions that did not relate to exceptionality has understandably created confusion about the purpose of EC and with determining the most appropriate scheme to apply under, particularly amongst applying clinicians.

To help improve clarity for stakeholders about the scope of funding pharmaceuticals outside of the Schedule, we propose listing on the Schedule as many treatments as possible for patient groups that are currently being funded through the Community EC mechanism for technical reasons only and which are not related to exceptionality. We propose these medicines either be listed in Section B of the Pharmaceutical Schedule or included in a new section of the Schedule (which would include prerequisites for prior-authorisation for funding if required).

The effect of this change would be that clinicians would no longer complete EC (or NPPA) funding applications for circumstances not related to exceptionality. If prior authorisation should be required to enable funded access to a treatment that cannot be listed on the Schedule, but which is not related to exceptionality, this would no longer occur through the NPPA scheme.

Consultation question:

17. What is your view of the proposal to stop funding treatments that are not related to exceptionality through EC?

Appendix 1: Purpose and criteria of the NPPA pathways

This appendix collates the details of the purpose and criteria for each proposed NPPA pathway, described by pathway.

Unique Clinical Circumstances

Some patients experience such a unique clinical condition or set of clinical circumstances that we would be unlikely to have considered these when deciding whether to list a pharmaceutical on the Schedule (a process which is designed for population groups). In these situations we would not expect there to be a patient population (e.g. this patient's clinical circumstances would differ from all others who are seeking this particular treatment). In order to meet our statutory obligations we need to assess whether we should fund treatments for individuals in this position. The Schedule process is not the most appropriate process for those patients who differ significantly to the population group(s) seeking the treatment. We therefore consider it would be more appropriate to assess funding for these individuals through the Unique Clinical Circumstances (UCC) pathway of the NPPA scheme.

A number of stakeholders who responded to our discussion document commented that EC should provide for conditions that are 'rare' or 'out of the ordinary'. Unique Clinical Circumstances provides for truly exceptional cases, although we recognise our proposal is not aligned with the views of those people who wanted to see the number limit on 'rarity' increased from 10. We consider the Urgent Assessment pathway is the appropriate avenue for considering the circumstances of those people who are part of a patient population (albeit a small one in some cases).

The proposed prerequisites for the UCC pathway of NPPA are that the patient:

1. has tried and failed all alternative funded treatments (or alternative treatments have been contraindicated), or has experienced such serious side effects with all other relevant funded treatments that treatment has been ceased; AND

2. is experiencing an indication or set of clinical circumstances that are unique to them and make their case different from all other patients with the same condition.

Applications meeting these prerequisites would be assessed according to PHARMAC's decision criteria. For more information on this process, please see section 4(b), page 18.

Urgent Assessment

There are some situations in which a patient with a serious clinical condition may experience a significant deterioration in health or lose the opportunity for a significant improvement in quality of life before assessment for listing treatment(s) not listed on the Schedule for the relevant indication is undertaken. This patient may be part of a group of patients with similar conditions. In our view, until the treatment(s) being sought has been subject to a full Schedule assessment for the relevant indication, it is fair to consider funding the treatment for individuals at risk of significant irreversible damage. This proposed change would be significant as it would see the balance shifted in favour of potentially providing funded access to treatments prior to full Schedule assessment.

The circumstances in which we would consider an application under the Urgent Assessment pathway of the NPPA scheme include where the patient's condition may be, but is not necessarily, 'rare' and where provision of the treatment is potentially life saving. These are situations that stakeholders who responded to the discussion document suggested an EC process should provide for. In addition, some medicines likely to be sought by people eligible for consideration under Urgent Assessment could be described as 'high cost' and 'highly specialised'. Improving access to these types of medicines has been a focus for some stakeholders, and this theme was also reflected in submissions.

Under the proposed Urgent Assessment pathway, once a Schedule assessment is completed and the pharmaceutical is either included on PHARMAC's prioritisation list or declined for funding, there would be no justification for considering funding for additional individuals through the NPPA scheme (unless unique clinical circumstances applied). These individuals would be in the same position as others awaiting the outcome of PHARMAC's Schedule funding process. Named patients who had received funded treatments prior to Schedule assessment would continue to receive funding following assessment, even if the pharmaceutical was not listed on the Schedule for the population, provided they continue to benefit from treatment and meet any conditions of funding at the time of approval.

The capacity for significant benefit, or the avoidance of significant harm, is key to this pathway. There must be a real expectation that there is a chance of significant recovery in health or maintenance of health where significant deterioration is otherwise inevitable.

The proposed prerequisites for the Urgent Assessment pathway of NPPA are that:

1. The patient has tried and failed all alternative funded treatments (or alternative treatments have been contraindicated), OR has experienced such serious side effects with all other relevant funded treatments that treatment has been ceased; AND

2. The indication or set of clinical circumstances may affect more than one patient (either currently or over time); AND

3. The patient has a serious clinical condition and not receiving the treatment prior to Schedule assessment would lead to either:
   - a significant deterioration in a serious clinical condition, OR
   - the patient would miss the opportunity for significant improvement in quality of life.

Applications meeting these prerequisites would be assessed according to PHARMAC's decision criteria. For more information on this process, please see section 4(b), page 18.

Hospital Pharmaceuticals in the Community

There are some circumstances in which it would be cheaper for a District Health Board (DHB) to fund an otherwise unfunded pharmaceutical for use in the community for an individual patient under its care, than to pay for the most likely alternative treatment or outcome. DHBs cannot do this without PHARMAC approval as the NZPHD Act requires DHBs to act consistently with the Pharmaceutical Schedule. We consider it fair that a process exists for DHBs to seek PHARMAC approval to be able to supply, and fund from their own budgets, these otherwise unfunded treatments.

The Hospital EC scheme currently provides this process. The proposed name change to 'Hospital Pharmaceuticals in the Community' aims to better reflect the proposed purpose of this

pathway, and to clarify that it is the provision of funding for treatments outside the Schedule that is exceptional not the clinical circumstances of the individual applicant. This clarification was sought by a number of stakeholders who responded to our discussion document.

The proposed prerequisites for assessment for funding under HPC are that:

1. The patient has tried and failed all alternative funded treatments (or alternative treatments have been contraindicated) or has experienced such serious side effects with all other relevant funded treatments that treatment has been ceased; AND

2. The application is for a DHB hospital to fund a treatment for use in the community for a patient under the care of a DHB hospital clinician (in-patient or out-patient); AND

3. The treatment is not a cancer treatment; AND

4. The treatment costs less for the DHB than the most likely alternative intervention or outcome; AND

5. The length of treatment is less than three months (noting that PHARMAC may approve treatments for longer than three months in compelling cases).

Applications meeting these prerequisites would be assessed according to PHARMAC's decision criteria. For more information on this process, please see section 4(b), page 18.

We propose trialling giving DHBs discretion to dispense, without prior PHARMAC approval, treatments that cost $500 or under and meet the prerequisites listed above.

Cancer treatments

As PHARMAC manages both the community and hospital funding of pharmaceutical cancer treatments, we consider it would be inappropriate for DHB hospitals to fund cancer treatments from their own budgets. Pharmaceutical cancer treatments would be eligible to be considered under the UCC and Urgent Assessment pathways, subject to meeting the relevant prerequisites.

Lower cost to the DHB than the alternative intervention or outcome

When considering the most likely alternative treatment, PHARMAC will generally not consider admitting the patient solely for the purpose of providing the treatment to be adequate to meet the prerequisite requirements for HPC.

Time limits on treatment length

The proposed criterion limiting the length of treatment to three months clarifies that funding treatments through the HPC pathway is not intended to be an alternative to a Schedule listing. Previously, where long-term treatments have been funded for a large number of patients through Hospital EC, PHARMAC has had difficulty when subsequently negotiating a Schedule listing because an alternative market had been created.

While we propose to limit the length of HPC funded treatments to three months, PHARMAC would be able to use its discretion to approve funding for longer time periods where this is clinically appropriate. We would expect to exercise this discretion only where a compelling case

is made as to why a community pharmaceutical treatment that is not listed on the Pharmaceutical Schedule should be provided to a patient for a longer time period.

Pharmaceuticals under $500

The lower the cost of the pharmaceutical treatment, the more likely it will be cheaper for the DHB compared with the alternative intervention or outcome. Recognising this, and that the application process is an administrative burden for applicants, we consider it desirable to trial setting a cost limit under which DHB hospital clinicians would be able to dispense, without PHARMAC approval, community treatments that are not listed on the Schedule. The proposed limit for the trial is $500 for the treatment course and the HPC prerequisites - including that treatment course is not longer than three months – would need to be met. Clinicians wishing to seek approval for funding a treatment for a longer duration would need to make an HPC application.

DHB hospitals wanting to participate in the trial would need to be able to provide PHARMAC with patient level data on those pharmaceuticals funded through HPC under $500 to enable PHARMAC to monitor prescribing. Such monitoring would be essential to ensure we could take action if it appeared that a market was being developed such that there was a risk that negotiation for listing on the Pharmaceutical Schedule could be undermined. If PHARMAC considered there was a risk with the funding of community treatments under HPC (whether under the limit or not), DHB hospitals would not be permitted to dispense these and we would decline all HPC applications for these products.

Appendix 2: Consultation questions

1. What are your views on the proposal that the purpose of EC is to provide a scheme for considering funding in those situations the Schedule process is not most appropriate?

2. What are your views on the proposal that the Named Patient Pharmaceutical Assessment scheme considers applications for funding for individuals rather than patient populations?

3. What are your views on the proposed purpose of the Unique Clinical Circumstances pathway?

4. What are your views on the proposed purpose of the Urgent Assessment pathway?

5. What are your views on the proposed purpose of the Hospital Pharmaceuticals in the Community pathway?

6. What, if any, additional situations do you consider the Schedule process does not best cater for that PHARMAC should consider under the Named Patient Pharmaceutical Assessment process?

7. What are your views on the proposed prerequisites for the Unique Clinical Circumstances pathway?

8. What are your views on the proposed prerequisites for the Urgent Assessment pathway?

9. What are your views on the proposed prerequisites for the Hospital Pharmaceuticals in the Community pathway?

10. What are your views on the proposal to assess Named Patient Pharmaceutical Applications against PHARMAC's decision criteria?

11. What are your views on the proposal that an NPPA application (other than within the UCC pathway) triggers PHARMAC's consideration of listing the treatment being requested on the Pharmaceutical Schedule?

12. Do you favour wider access with the appearance of inconsistent decisions that this may bring, or a more restrictive but consistent approach?

13. What are your views on removing the distinction between cancer and community treatments under the proposed NPPA scheme?

14. What are your views on the proposed approach to funding for treatments approved under the NPPA scheme?

15. What are your views on the proposed operational arrangements for the NPPA scheme?

16. What information do DHB hospitals routinely collect and how would this satisfy the information needs we have identified as necessary from DHB hospitals wishing to participate in the trial described in section 6(a)(ii).

17. What is your view of the proposal to stop funding treatments that are not related to exceptionality through EC?