CONSULTATION

PHARMAC
Pharmaceutical Management Agency

# Contestable fund for medicines for rare disorders

PHARMAC will be running a commercial process to fund medicines for rare disorders – a request for proposals (RFP). A draft of the RFP is attached to this letter. The objective of this process is to improve patients' access to medicines for rare disorders.

The release of the draft RFP and this consultation letter follows a discussion on the scope and shape of the fund, and the type of medicines it might cover. We now would like your feedback on the contents and process outlined in the draft RFP before we proceed to the next stage, which would be actively seeking proposals from pharmaceutical suppliers.

Your feedback is important to us. You can provide feedback by answering the questions that follow, or by providing any other views or information to us. We would prefer this feedback to be in writing, and provided to us at:

- contestablefund@pharmac.govt.nz or
- PHARMAC
  PO Box 10254
  The Terrace
  Wellington 6143

Your feedback should be provided to PHARMAC by 25 July 2014.

**Key points**

- Up to $5 million is available for investment each year, from the 2014/15 financial year onwards, to fund medicines for rare disorders from this contestable process.
- Any medicines funded through this process would be listed on the Pharmaceutical Schedule. We would expect to continue funding medicines for any patients receiving them through this process, for as long as they continued to benefit according to transparent and measurable clinical criteria. The measurement of this benefit may be subject to Special Authority criteria or assessed via a Panel or similar process.
- "Rare" would be defined as the long term disorder having a prevalence of one in 50,000 people. This would mean that up 90 New Zealanders might be eligible for treatment with each medicine funded as a result of the RFP in any one year.
- PHARMAC would be willing to consider a wide range of proposals from suppliers, including individual or multiple product proposals, and risk-sharing proposals to keep expenditure within $5 million per annum.
- Proposals could be accepted for medicines not yet registered in New Zealand, but not for medicines in the experimental phase of development. Medicines would need to be registered in New Zealand before a Pharmaceutical Schedule listing occurred.

- PHARMAC has established an expert Subcommittee of clinicians that will provide advice on whether any criteria for patients to be eligible for funding are clinically meaningful and can be implemented.

- PHARMAC would assess proposals against its statutory objective using its Operating Policies and Procedures (OPP) at the time.

## Background

On 8 April 2014 we released a discussion document on the topic of high cost medicines for rare disorders. We announced our intention to test out a contestable fund to improve access to potentially effective medicines for people with rare disorders and sought comments from the public and industry to help iron out some of the detail of how the fund would operate. You can find that discussion document on our website: http://www.pharmac.health.nz/medicines/how-medicines-are-funded/medicines-for-rare-disorders/. A summary of feedback received in response to the discussion document can be found in Section Four (p11).

We have decided that we will progress a Request for Proposals (RFP) to seek funding bids from suppliers, and we are now consulting on a draft version of the RFP. This is in line with our aim of making funding decisions in early 2015.

## Purpose of this document

This consultation document seeks your feedback on the proposed prerequisites for the RFP (Section One, p4).

The letter also provides information on the commercial aspects of the proposed RFP process, outlines implications for Named Patient Pharmaceutical Assessment applications and seeks your feedback on proposed changes to the confidentiality clause in the RFP; and

Expressions of Interest are also sought from suppliers based on a draft version of the RFP (Section Three, p10).

## Potential funding and process

PHARMAC has identified funding of up to $5 million that will be available each year, from money that had previously been budgeted for the Named Patient Pharmaceutical Assessment (NPPA) Policy. The funding has become available because PHARMAC has listed 42 medicines (to date) on the Pharmaceutical Schedule that were previously being routinely assessed for funding through the NPPA Policy, reducing the expenditure budgeted for the NPPA Policy.

With the funding available, PHARMAC has decided to trial a new commercial approach to seek bids for funding for medicines for rare disorders.

We would require suppliers to submit bids that could be managed from within the up to $5 million we have available each year. This may require suppliers to propose a form of risk sharing or to cap expenditure. Pharmaceutical suppliers would also be able to define the patient population and to propose eligibility criteria for patients to access funded treatment. We will seek clinical advice on whether any patient population and eligibility criteria proposed are clinically meaningful and whether they can be implemented in practice.

Bids that meet the prerequisites outlined below would be evaluated and, depending on the number of bids received, prioritised using the decision criteria (or equivalent) set out in

PHARMAC's then current OPP. PHARMAC would then decide which, if any, bids it would fund in light of the decision criteria (or equivalent) and our Statutory Objective. Successful bids would be listed on the Pharmaceutical Schedule, using Special Authority criteria, restrictions, or access via a Panel or similar process if necessary.

Funding for listed treatments would be ongoing. People who receive medicines funded through the contestable fund would continue to have them funded for as long as they continued to benefit, in accordance with transparent and measurable clinical criteria.

PHARMAC has established a new Subcommittee of the Pharmacology and Therapeutics Advisory Committee (PTAC) – the Medicines for Rare Disorders Subcommittee - whose role is to provide objective clinical advice to PTAC and/or PHARMAC on medicines for rare disorders. The Subcommittee will remain in existence for 12 months to support this competitive process. The Subcommittee has advised PHARMAC that it considered the one in 50,000 prevalence was appropriate to use as a measure of rarity for the New Zealand population, and gave advice on the proposed prerequisites included in the draft RFP.

We also expect to seek advice from the Subcommittee on the bids received, including advice on:

- Whether bids meet the fund's prerequisites.
- The quality of the clinical evidence (particularly regarding health need and treatment efficacy) submitted or otherwise available for any bids for medicines that have not already been assessed by PTAC.
- Advice on any bids for medicines that have already been assessed by PTAC, to account for new evidence and / or pricing changes.
- Clinical acceptability and measurability of possible or bidder proposed eligibility criteria and on-going eligibility for funding.

Decisions on funding will be made by the PHARMAC Board or its delegate.

## Section One: Proposed prerequisites for the RFP

### Prerequisites for medicines for rare disorders

We are proposing to include a set of prerequisites in the RFP to assess whether the bids we receive are eligible to be considered. If a bid meets the prerequisites it would then be assessed, alongside all other the eligible bids, using the decision criteria (or equivalent) set out in PHARMAC's current OPP at the time. Products included in bids which do not meet the pre-requisites would still be eligible for consideration for Schedule listing, or NPPA funding for individual patients, in line with PHARMAC's usual decision making processes.

The proposed prerequisites are listed below, followed by a description of each of the prerequisites and some consultation questions.

*Disorder related*

1. There is a rare[1] but clinically defined long-term disorder that is identifiable with reasonable diagnostic precision.
2. Epidemiological and other studies provide evidence acceptable to PHARMAC[2] that the disorder causes a significant reduction in either absolute or relative age-specific life expectancy or quality of life, for those suffering from the disorder[3].

*Treatment related*

3. The medicine is regarded as a proven therapeutic modality for an identifiable patient population[4] i.e. the medicine has been approved by Medsafe or an international regulatory authority[5] for the identified indication.
4. There is evidence acceptable to PHARMAC[6] that the medicine is likely to be clinically effective for the identified patient population[4].
5. The patient's absolute or relative age-specific life expectancy or quality of life could be substantially improved as a direct consequence of the treatment[7].

*Alternatives related*

6. The medicine is not registered for the treatment of another, non-rare disorder, or if it is, the cumulative prevalence across all the indications still falls within the definition of rare[8].
7. There is no suitable comparable[9] alternative treatment on the Pharmaceutical Schedule.
8. There is no suitable[9] funded alternative non-drug therapeutic modality for the rare disorder.

[1] Rare is defined as an identifiable and measurable patient population with a prevalence of 1:50,000 or less.
[2] On the basis of advice from PTAC and / or the RAD Subcommittee of PTAC.
[3] As measured by absolute or proportional QALY loss.
[4] The definition of the patient population must be clinically meaningful (not arbitrary) and must treat patients with the same clinical circumstances equally.
[5] Regulators that are recognised by Medsafe for the purposes of an abbreviated approval process, as listed on page 38 of - http://www.medsafe.govt.nz/regulatory/Guideline/Full%20-%20NZ%20Regulatory%20Guidelines%20for%20Medicines.pdf
[6] On the basis of advice from PTAC and / or the RAD Subcommittee of PTAC.
[7] As measured by absolute or proportional QALY gain.
[8] Bidders would be required to reveal their overseas approved indications and their phase three development programme.
[9] Suitable is defined as a treatment that provides a comparable health outcome to the medicine under consideration, for the patient population under consideration

**Explanation of prerequisites**

*Prerequisites one and six – rarity*

There is no universally-accepted definition of what 'rarity' is. A condition may be considered rare in one part of the world, or in a particular group of people, but be considered common elsewhere.

We are proposing to define a rare disorder as one that affects one person for every 50,000 people in the general population. This definition would mean there are currently up to 90 people across the whole of New Zealand that have each rare disorder. This is consistent with the definition used in the United Kingdom (UK) of an "ultra-orphan" disease being 1:50,000.

We also propose that the prevalence definition would apply to ongoing conditions - a condition lasting longer than 12 months.

Suppliers might wish to seek to limit the total number of patients eligible for funding to a number which offers a sufficient return on investment, taking into account the fixed funding available. We therefore propose that suppliers would be able to identify a sub-set of people with the disorder who would be eligible for funding, as long as the sub-set is distinct and clinically meaningful, and the total number of people with the disorder still meets the 1:50,000 prevalence criterion.

| Consultation Question: What do you think of the proposed rarity definition? |
|---|

*Prerequisite 2 – disease severity*

Under the fund, not only must the disorder be rare but it must cause a significant reduction in either absolute or relative age-specific life expectancy or quality of life, for those suffering from the disorder.

We are proposing to measure severity by any reduction in a person's Quality Adjusted Life Years (QALYs) that is due to the disorder. Changes in QALYs measure how much the disorder shortens a person's life expectancy (loss of quantity of life) and how much it reduces the quality of life. Further information on the QALY measure is available in past PHARMAC annual reviews[10].

| Consultation Question: Do you think that the RFP should be limited to medicines that treat disorders that cause a significant reduction in either life expectancy or quality of life? Why or why not? |
|---|

*Prerequisite 3 – registration*

The bid must be for a medicine to treat the rare disorder (i.e. only medicine funding proposals will be considered).

The treatment must be a proven therapeutic modality; it should not include experimental or medicines still in trial. To this end, we propose only considering bids for medicines that have been registered by Medsafe or an international regulator recognised by Medsafe. This would provide an appropriate level of assurance of the product's safety and efficacy. It is our

[10] PHARMAC Annual Review 2010/11. Wellington: PHARMAC, 2011. http://www.pharmac.health.nz/assets/annual-review-2011.pdf (pages 12-13, article by Prof Anthony Harris)

expectation that, prior to being listed on the Pharmaceutical Schedule, medicines funded via the contestable fund would need to be registered by Medsafe or have been submitted for registration with Medsafe. If we receive a bid for an unregistered product, we have the option to initiate a Pharmaceutical Schedule listing application outside of the contestable fund process.

You can find a list of international regulators recognised by Medsafe on page 38 of Medsafe's Regulatory Guidelines for Medicines at http://www.medsafe.govt.nz/regulatory/Guideline/Full%20%20NZ%20Regulatory%20Guidelines%20for%20Medicines.pdf.

| Consultation Question: Do you think prerequisite 3, which limits eligible bids to those that have either been approved by Medsafe or an international regulator recognised by Medsafe, is appropriate? Why or why not? |
|---|

*Prerequisite 4 and 5 – effectiveness*

We propose that there needs to be evidence "acceptable to PHARMAC" that the medicine is effective. We need to reach a threshold of confidence, and it may not be possible for us to determine whether this threshold is reached for completely new medicines that have not already been assessed by the Pharmacology and Therapeutics Advisory Committee (PTAC) (whether they are registered or not) within the time we have available for the RFP.

To allow products which may meet all the prerequisite criteria except for the 'evidence acceptable to PHARMAC' criteria, we are requesting expressions of interest. This would allow suppliers of medicines not previously assessed by PTAC to alert PHARMAC to the potential of a proposal, thus allowing us to seek advice from PTAC. We will also have the option to initiate a Schedule listing application for any medicines that we are unable to fully consider within the RFP timelines.

Suppliers seeking funding would need to demonstrate that the medicine could significantly affect the natural history of the disorder it treats. We would need evidence that the medicine is likely to be clinically effective and would result in a significant extension in life expectancy or quality of life compared with currently funded treatments.

| Consultation Question: Do you think prerequisites 4 and 5, relating to the effectiveness of the medicine, are appropriate? Why or why not? |
|---|

*Criteria 7 and 8 – alternative treatments*

We propose excluding medicines for which there is already a comparable treatment funded. We propose that before a medicine is excluded on the basis of prerequisites 7 and 8, the safety and efficacy of the alternative funded treatment must be comparable to the medicine for which we have received a bid, in terms of either absolute or relative age-specific life expectancy or quality of life. For example, this would mean that best supportive care typically would not be considered to be a comparable alternative treatment.

| Consultation Question: Do you think prerequisites 7 and 8, relating to the availability of suitable alternatives, are appropriate? Why or why not? |
|---|

## Section Two: Commercial aspects and impacts on NPPA

### Explanation of the commercial aspects of the RFP

An RFP is a procurement process similar to tendering but allowing more flexibility. It is an invitation for suppliers to submit a proposal to supply a specific medicine or medicines. PHARMAC uses RFPs to generate competition between suppliers for medicine subsidies, when tendering is not appropriate – often because there are non-price elements that may vary between offers from different suppliers. We think an RFP is the best competitive tool to use in this case, because comparing bids for medicines for different rare disorders (treating patients with differing health needs) is extremely complex and does not lend itself to a tender process where simple pricing bids are received in response to pre-set funding criteria.

Some of the responses to the discussion document expressed concerns with PHARMAC's 'standard' RFP techniques such as sole supply and bundling. Explanations of these terms and how we propose to use them in this process are outlined below.

*Sole subsidised supply*

This allows suppliers who have competitors for their pharmaceutical to be the only brand or type of their medicine that is subsidised. This approach gives suppliers maximum incentive to offer their most competitive pricing, because they know they will have a guaranteed market for a set period of time.

Sole supply proposals will be considered in this process.

*Bundling*

We propose allowing companies to make bundled offers. A bundle is where a company is able to supply more than one pharmaceutical, including a new pharmaceutical, and offers savings on other products to reduce the overall cost of listing a new product. Not all companies have large portfolios of products, so in order to improve comparability we plan to ask companies to also submit an individual proposal for each medicine for a rare disorder that meets the prerequisites which is included in the bundle.

Each bid, whether bundled or not, would be assessed using PHARMAC's decision criteria (or equivalent) set out in PHARMAC's then current OPP (which require that we look at more than just the overall budgetary impact), and using PHARMAC's overall statutory objective of securing the best health outcomes that are reasonably achievable from pharmaceutical treatment and from within the amount of funding provided.

*Caps, rebates, or other expenditure risk sharing mechanisms:*

Financial risk sharing arrangements such as volume-related prices where expenditure above an agreed level is met by the supplier are relatively common in New Zealand as are mechanisms like discounted prices and rebate arrangements (where a price lower than the published price is negotiated). These types of arrangements mean competitive pricing can be offered which ensure the New Zealand taxpayer is not exposed to undesired total costs, while suppliers are able to protect their sales prices in other countries. We would consider proposals for pricing discount mechanisms, or other risk-sharing arrangements, as part of the RFP process.

We wouldn't consider a risk sharing proposal that sets a fixed limit on the number of patients eligible for funding. This is because we consider that all patients with a similar clinical situation must be able to access the funded treatment.

*Proposals with eligibility criteria*

We propose that a supplier can offer PHARMAC a commercial proposal which includes clinically acceptable and measurable eligibility criteria. These could include criteria for beginning treatment, or for ending treatment if it was no longer effective. These criteria would target funding to patients who would benefit most from the therapy. This type of approach improves the cost effectiveness of any proposal, as the funding would target the patients with the greatest capacity and likelihood to benefit. This would also enable suppliers to estimate the patient numbers for the purpose of calculating expenditure risk sharing proposals.

PHARMAC would seek advice on any eligibility criteria proposed by a supplier from the Medicines for Rare Disorders Subcommittee of PTAC.

**Proposed amendment to Schedule 2, clause 5(j) of the Draft RFP**

PHARMAC's standard terms for an RFP state that proposals and information exchanged between suppliers and PHARMAC in any negotiations relating to proposals, excluding information already in the public domain, are considered "Confidential Information".

As there is likely to be significant public interest in this RFP, PHARMAC proposes making an amendment to expressly state that the following information is not "Confidential Information":

1) The existence of a proposal; and/or

2) The name of the supplier who submitted a proposal.

This would ensure that PHARMAC is able to provide information regarding the number of proposals received and/or the companies who submitted proposals.

Other information falling within the definition of "Confidential Information" in the draft RFP would remain "Confidential Information".

| Consultation Question: Do you consider it appropriate to state in the RFP that the existence of proposals is not confidential? Do you consider it appropriate for PHARMAC to secure the ability to make the name of the suppliers submitting proposals public? |
|---|

**Implications for Named Patient Pharmaceutical Assessment (NPPA) applications**

One of the prerequisites of the NPPA policy is that the medicine has not already been considered through the Schedule process. We have given some thought to the implications of this RFP for future NPPA applications, and whether a new medicine that has been considered through this RFP bid process (but has not yet been considered via the Schedule Funding application process) should be considered as having been 'considered', 'prioritised', or 'declined' for the purposes of future NPPA applications.

Bids received through this RFP process should not be regarded as having been 'considered' or 'prioritised for Schedule listing', simply because a bid has been submitted and evaluated.

We have identified three possible scenarios:

1. The treatment **has** been considered by PTAC and prioritised or declined by PHARMAC for funding. In this case the treatment is *already ineligible for NPPA consideration*, unless the patient's clinical circumstances are different to those considered by PTAC or PHARMAC uses its wider discretion to consider funding. This will continue to be our interpretation of the NPPA Policy, and we suspect will be the situation for the majority of the RFP bids we receive.

2. The treatment **has not** been considered by PTAC and prioritised or declined by PHARMAC for Schedule listing, and it **is not** progressed for funding as part of this RFP because it does not meet the RFP prerequisites or we are unable to gather sufficient clinical evidence. In this case applications *would be eligible to be considered under the NPPA policy* provided other NPPA Policy prerequisites are met. PHARMAC could self-initiate a Schedule application, and NPPA applications would be able to be considered until a Schedule application was prioritised or declined by PHARMAC for listing, which is the normal process under the NPPA Policy.

3. The treatment **has not** been considered by PTAC and prioritised or declined for funding, and it **is** listed in the Pharmaceutical Schedule, as a result of this RFP, with entry and exit criteria. In this case a NPPA application for a patient who did not meet the Schedule entry and exit criteria *might be eligible to be considered under the NPPA policy* provided that PHARMAC determines that the patient is not part of the group that was actively considered when the decision to fund the treatment was made, and provided that all other NPPA Policy prerequisites are met. This is consistent with the approach followed by PHARMAC when assessing NPPA applications for medicines currently listed on the Pharmaceutical Schedule with Special Authority criteria or restrictions.

The rationale for the proposed approach to scenario two is that the bid has not been given due consideration for Schedule listing, and unlike a standard Schedule listing application, the RFP bid does not continue to be an active funding application or option for PHARMAC.

In scenario three it is likely that PHARMAC would, at the time of funding (under the RFP), initiate a funding application for the wider or different population group. This would ensure that the entire population is eventually considered for funding (although there may be a time window in which prioritisation has not occurred so a NPPA application could be considered).

## Section Three: Expressions of interest

As a part of this process PHARMAC would welcome any expressions of interest from suppliers who would be interested in pursuing a listing agreement with PHARMAC. PHARMAC intends to release the Request for Proposals (a competitive process) which could result in an exclusive listing agreement (or agreements).

PHARMAC has identified products that in our opinion might meet some or all of the prerequisites for consideration under the draft RFP. PHARMAC is also interested in receiving expressions of interest from suppliers of other pharmaceuticals that in the supplier's opinion would meet the prerequisites for consideration of funding.

Given that PHARMAC may require PTAC assessment of pharmaceuticals if this has not already occurred, early notification from the supplier, via an expression of interest, would help PHARMAC to ensure time could be made for this assessment at a PTAC meeting. Provision of information relating to registration status or provisional registration timelines would be useful in any expressions of interest.

Please contact contestablefund@pharmac.govt.nz regarding an expression of interest. Expressions of interest must be received by PHARMAC by 25 July 2014.

## Section Four: Feedback Received in Response to the Discussion Document

Following publication of the discussion document on high cost medicines for rare disorders, we met with three stakeholders (one person with a rare disorder, one patient representative group, and one health professional organisation) and received eight written submissions (from members of the public, clinicians, clinical organisations, health professional organisations, and patient representation organisations). The discussion document was also an agenda item at a Stakeholder Consultation event that was held in Wellington on 15 April 2014.

A number of submissions we received in response to our Decision Criteria and NPPA reviews also included feedback on the details of the contestable fund, and we have also taken that feedback into account. Many submitters may have chosen to wait for the draft RFP to be published before providing their feedback. The submissions discussed below are those that were received before 27 June 2014.

Almost all submitters (clinicians, clinical groups and members of the public) were supportive of the concept and felt it was worth progressing further. One submitter applauded the innovation and intent.

One submitter said that ring-fencing funding for rare disorders would result in more equitable outcomes because it would avoid treatments for rare disorders having to compete with inexpensive medicines that can be provided to large numbers of people while a different submitter opposed the separate fund on the ethical grounds of reduced equity of access. Another submitter suggested that the number of people likely to be treated should be a factor in all funding decisions, rather than seeking to dichotomise conditions into rare and not-rare, which could disadvantage those with “almost rare” diseases.

All submitters who specifically commented on the proposed prerequisites were supportive of their inclusion. The table below sets out some of the specific feedback received so far, and our response.

| Feedback Received | Response |
|---|---|
| One submitter questioned whether the publicly funded system sufficiently supports education and IVF and embryo selection, or whether this would be a cost effective area for further development. | While noted, this suggestion is outside of the scope of this project and PHARMAC’s remit. |
| One submitter suggested that PHARMAC should consider establishing a type of insurance fund that provides coverage to patients up to a certain amount (for rare medications), or that PHARMAC could coordinate the many crowd funding initiatives that are currently undertaken on social media and the mainstream media. | While noted, this suggestion is outside of the scope of this project and PHARMAC’s remit. |

| Feedback Received | Response |
|---|---|
| One submitter noted that if the fund has high take-up, it might become inaccessible to new medicines/patients, as patients whose treatments are funded early on would continue on treatment for many years. | Any medicines funded through this process would be listed on the Pharmaceutical Schedule, so any people meeting access criteria would also receive funded treatment. People who receive medicines funded through the contestable fund would continue to have them funded for as long as they continued to benefit.<br><br>PHARMAC is trialling using a contestable fund, to see whether it is effective in helping improve access to patients seeking medicines for rare disorders. PHARMAC will be evaluating the process to look at whether the RFP achieved what we wanted it to achieve (better commercial price offers) and whether it's a process that we may want to run again in the future. |
| Some submitters commented on the perceived inconsistency between the contestable fund proposal and PHARMAC's decision criteria consultation document, which states that PHARMAC proposes to use the same decision-making framework for all funding decisions.<br><br>Another submitter suggested that PHARMAC should not use different decision criteria for medicines for rare disorders. | The proposed RFP process will trial an alternative commercial approach within the usual constructs of PHARMAC's funding: capped budget, health need and cost-effectiveness assessment, and relative prioritisation.<br><br>We think that it is important that the decision criteria (or equivalent) that are used for all PHARMAC funding decisions are also used to consider the bids we receive through the RFP. The proposed prerequisites will determine whether a bid can be considered for funding from the contestable fund. Then all eligible bids will be evaluated and (if required) prioritised using PHARMAC's decision criteria (or equivalent) set out in PHARMAC's current OPPs at the time[11]. |
| One submitter supported the idea of having entry and exit criteria for expensive medicines, but noted that for very rare disorders with only one or two patients, it may be impractical and impose unnecessary bureaucracy to fund these via the RFP rather than NPPA<br><br>Another submitter commented that where there are medicines with clear life benefits, where there is no other option for treatment, these should not be included in a contestable fund and should instead be listed on the Pharmaceutical Schedule with restricted prescribing rights | Any medicines funded through this process would be listed on the Pharmaceutical Schedule, and if eligibility criteria are used these would be reflected via Special Authority or Panel mechanisms. If a medicine was listed through the RFP process, it would be clear whether or not a patient would be eligible for funding, rather than needing to make individual NPPA applications with uncertainty of outcome. |

[11] Note that PHARMAC's decision criteria are currently under review, you can find more information on our website: http://www.pharmac.health.nz/about/operating-policies-and-procedures/decision-criteria-consultation

| Feedback Received | Response |
|---|---|
| One submitter suggested applicants should be required to make a case for the clear delivery of substantial health benefits in order to be funded from the pool. | We propose to include a prerequisite that the disorder causes a significant reduction in life expectancy or quality of life, and that the medicine is likely to be clinically effective. |
| Three submitters commented that the $5 million proposed was insufficient, and a range of ideas were also presented about how the fund could be increased over time | We note the views of submitters on the size of the fund.<br><br>As explained in the discussion document, the projected expenditure on NPPA has reduced as a result of Schedule listings so we anticipate that in 2015/16 we have up to $5 million available to spend on other things. This would be long-term funding, so total expenditure could be $25 million over the first five years. |
| One submitter suggested that the pool should not include experimental or trial medicines | We propose to include a prerequisite that the treatment must be regarded as a proven therapeutic modality and that it has been approved by Medsafe or an international regulatory authority. |
| One submitter said that suppliers shouldn't be able to propose entry and exit criteria | We would seek advice from the Medicines for Rare Disorders Subcommittee of PTAC on whether any criteria proposed are clinically meaningful, can be implemented practically and are not arbitrary. |
| One Submitter noted the potential 'moral hazard' if the fund was spread over two or three groups of people but not the others, given that for all of the disorders under consideration there is no other effective treatment currently available. | The Draft RFP allows suppliers to submit more than one proposal, which would allow them to target patients most likely to benefit. This could result in a wider range of products potentially being funded as a result of the process.<br><br>As outlined above, PHARMAC will be evaluating the process to look at whether the RFP achieved what we wanted it to achieve (better commercial price offers) and whether it's a process that we may want to run again in the future. |
| One submitter commented that some rare diseases are treated with commonly used drugs, and noted that if a medication is to be used for one disorder it may be possible to have it used for other less rare disorders or milder cases as the incremental cost of supplying additional drug product is not usually high. | For this reason we are proposing to include a prerequisite that states that the medicine cannot be indicated for the treatment of another non-rare disorder. |

| Feedback Received | Response |
|---|---|
| One submitter questioned why PHARMAC did not just increase funding for the Named Patient Pharmaceutical Assessment (NPPA) policy instead of establishing the fund. | The contestable fund would result in medicines being listed on the Pharmaceutical Schedule. If a medicine was listed through the contestable fund, all eligible patients would receive it without needing to make individual applications with uncertainty of outcome.<br><br>Medicines funded under NPPA are not contracted for by PHARMAC and therefore there is not price or supply certainty.<br><br>The NPPA policy does not promote competition among suppliers, which is what the approach we are currently looking at would do. Increased competition could lead to improved outcomes for patients, through lower prices enabling PHARMAC to provide funding. |
| One submitter noted that allowing bundling deals could disadvantage patients seeking treatments supplied by companies that only have one product | We propose to require suppliers to include individual bids if they intend to include a bundled proposal.<br><br>Each bid (bundled or not) would be assessed using PHARMAC's nine decision criteria (which require us to look at more than just the overall budgetary impact), and overall statutory objective of securing the best health outcomes that are reasonably achievable from pharmaceutical treatment and from within the amount of funding provided. |
| One submitter noted that it is important that the fund does not only fund products that are already routinely being approved via NPPA. | The NPPA Policy considers individual circumstances. An approval for one patient does not mean that all patients seeking funding for the same medicine would be approved (unless their clinical circumstances were the same in which case this generally will occur). The rare disorders RFP is seeking proposals to treat patient populations with similar clinical circumstances without the need for individual consideration. We cannot guarantee that a product that has been approved under NPPA for an individual would not be funded via this scheme, but any funding decision would be for every patient meeting the entry and exit criteria. This would represent a widening of access.<br><br>If a patient was already being funded via an NPPA approval, and they met the entry and exit criteria agreed via the RFP process, their NPPA approval along with their pre-committed funding could be changed to a Pharmaceutical Schedule approval. This would maintain the up to $5 million of new spending proposed. |