

8 July 2014

Dear Supplier

DRAFT REQUEST FOR PROPOSALS – SUPPLY OF MEDICINES FOR RARE DISORDERS

PHARMAC is establishing a contestable fund for medicines for rare disorders. PHARMAC intends to invite proposals later in 2014 for the supply of medicines for rare disorders.

This request for proposals (**RFP**) letter outlines:

- the types of pharmaceutical for which PHARMAC is requesting proposals and sets out the background to the RFP and the types of proposals sought (schedule 1);
- the process that PHARMAC expects to follow in relation to the RFP (schedule 2);
- information about the estimated size of the current subsidised market for the pharmaceutical (schedule 3).

Schedule 1: Pharmaceutical, background to RFP and types of proposals sought

1. Pharmaceutical

PHARMAC is interested in considering proposals from suppliers of medicines for rare disorders for the funding and listing of their products in the Pharmaceutical Schedule.

2. Background to RFP

PHARMAC is trialling a new approach to introduce competition into the area of medicines for rare disorders. We are offering a contestable fund of up to \$5 million per annum (on-going) for these medicines, our aim being to improve funded access to effective treatments for rare disorders by incentivising pharmaceutical suppliers to make competitive pricing offers.

3. Proposed parameters and pre-requisites for types of proposals sought

PHARMAC is interested in receiving proposals for medicines for rare disorders which meet the following pre-requisites.

Prerequisites for medicines for rare disorders:

For an explanation of these prerequisites please see the follow link.

Disorder related

1. There is a rare¹ but clinically defined long-term disorder that is identifiable with reasonable diagnostic precision.
2. Epidemiological and other studies provide evidence acceptable to PHARMAC² 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 disorder³.

Treatment related

3. The medicine is regarded as a proven therapeutic modality for an identifiable patient population⁴ i.e. the medicine has been approved by Medsafe or an international regulatory authority⁵ for the identified indication.
4. There is evidence acceptable to PHARMAC⁶ that the medicine is likely to be clinically effective for the identified patient population⁴.
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 treatment⁷.

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 rare⁸.

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.

7. There is no suitable comparable⁹ alternative treatment on the Pharmaceutical Schedule.
8. There is no suitable⁹ funded alternative non-drug therapeutic modality for the rare disorder.

4. PHARMAC is willing to consider the following types of proposals:

- sole subsidised supply, provided sole supply extends no later than 30 June 2018;
- caps, rebates, or other expenditure risk sharing mechanisms;
- cross-deal or bundling arrangements in respect of more than one chemical entity, therapeutic group or sub-group, when at least one of the pharmaceuticals involved is for the treatment of a rare disorder;
- proposals with clinically acceptable and measurable entry and exit criteria

Please note:

- If you wish to submit a cross-deal or bundled proposal, you must also submit an individual proposal solely related to each pharmaceutical within the bundle that treats a rare disorder.

PHARMAC is not willing to consider the following types of proposals:

- caps, rebates, or other expenditure risk sharing mechanisms where the cap, rebate or other expenditure risk sharing mechanism is based on a restricted number of patients. Patients with similar clinical circumstances must be eligible for the same funding.

Subject to the above, PHARMAC is open to considering any other types of proposals you may wish to put forward.

⁹ Suitable is defined as a treatment that provides a comparable health outcome to the medicine under consideration, for the patient population under consideration

Schedule 2: RFP process

PHARMAC expects to follow the process set out below in the sequence indicated.

1. Submission

- (a) You may submit more than one proposal. Each proposal will be considered as a separate proposal.
- (b) Proposals must be submitted by [date to be determined]. Late proposals will only be considered at PHARMAC's discretion.
- (c) You cannot withdraw your proposal, once submitted, while the RFP process is continuing.
- (d) All proposals must be submitted to PHARMAC by email or electronic copy.

2. Evaluation

- (a) Following the deadline for submitting proposals an Evaluation Committee comprising PHARMAC staff will evaluate each proposal to assess whether bids are eligible to be considered (i.e. do they meet the pre-requisites) and then select its preferred proposal(s).
- (b) Not all (or indeed potentially not any) eligible bids would receive funding.
- (c) The basis on which the Evaluation Committee will evaluate proposals, and the weight to be given to the criteria and other matters that it considers, are to be determined by the Evaluation Committee at its sole discretion. The matters to be taken into account by the Evaluation Committee will, however, include:
 - (i) the decision criteria (or equivalent) set out in PHARMAC's then current Operating Policies and Procedures (**OPPs**), as published on PHARMAC's website (www.pharmac.govt.nz), to the extent applicable;
 - (ii) any clinical advice from PTAC or its relevant sub-committees; The Subcommittee is likely to provide advice to PHARMAC on proposals received, with respect to
 - (A) Whether bids received meet the fund's pre-requisites (if required).
 - (B) 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 (if required).
 - (C) Advice on any bids for medicines that have already been assessed by PTAC, to account for new evidence and / or pricing changes.
 - (D) Clinical acceptability and measurability of possible or bidder proposed entry and exit criteria and on-going eligibility for funding (if required).
 - (iii) any other matters that the Evaluation Committee considers to be relevant (provided that PHARMAC will notify such matters and allow an opportunity for submitters of proposals to address them).
- (d) Each proposal will be evaluated on the basis that the price offered, the expenditure entailed, and any other terms included in the proposal, are the best that the

supplier is able to offer. If you do not put forward your best terms you risk having your proposal excluded at the evaluation stage.

- (e) PHARMAC is not bound to select the lowest priced proposal or any proposal.

3. Negotiation

- (a) PHARMAC may negotiate with the submitter(s) of one or more preferred proposals, in the latter case whether or not the acceptance of such supplier's proposal would exclude acceptance of any other proposal.
- (b) Negotiations will proceed on the basis that PHARMAC's standard terms and conditions for supply of pharmaceuticals, which are available on request from PHARMAC, will apply.
- (c) Given that PHARMAC expects your proposal to be the best you can offer, PHARMAC does not intend to initiate negotiation with you on price. However, PHARMAC does not exclude the possibility that the final price agreed will be different from the price put forward in your proposal, as a result of the impact that other negotiated terms may have on price.
- (d) PHARMAC may negotiate and enter into a provisional agreement with a preferred supplier(s) on whatever special terms, in addition to PHARMAC's standard terms and conditions, PHARMAC considers appropriate.
- (e) If PHARMAC and the supplier(s) are unable to reach a provisional agreement within what PHARMAC considers to be a reasonable time, PHARMAC may terminate those negotiations and negotiate, or continue to negotiate, with a different supplier(s).

4. Consultation and approval

- (a) Any provisional agreement will be conditional on consultation with suppliers and other interested parties, to the extent PHARMAC considers consultation to be necessary or appropriate, and on Board approval (or approval by the Board's delegate acting under delegated authority).
- (b) PHARMAC will not consider any counter-offers received during consultation.
- (c) The provisional agreement and responses to consultation will be considered by PHARMAC's Board (or by the Board's delegate acting under delegated authority) in accordance with the decision criteria in PHARMAC's then current OPPs.
- (d) If the Board or its delegate does not approve a particular provisional agreement, then PHARMAC may initiate negotiations and negotiate, or continue to negotiate, for a provisional agreement with any other supplier(s).
- (e) The RFP process will be complete once PHARMAC has notified suppliers of either:
 - (i) the Board's or its delegate's final decisions in relation to the RFP; or
 - (ii) the termination of the RFP process.

5. Miscellaneous

- (a) PHARMAC reserves the right:
 - (i) to make such adjustments to the above RFP process as it considers appropriate, at any time during the process, provided that it notifies suppliers affected by those changes;
 - (ii) not to accept any proposal;
 - (iii) to seek clarification of any proposal;
 - (iv) to meet with any supplier in relation to its proposal;
 - (v) to enter into an agreement or arrangement that differs in material respects from that envisaged in this RFP letter;
 - (vi) to suspend this RFP process. For example, if during the RFP process (and before a provisional agreement is entered into) it becomes apparent to PHARMAC that further consultation is appropriate or required we may suspend the RFP process in order to consult. In this situation we may ask you to adapt and resubmit your proposal in light of consultation, or alternatively we may request that new proposals be submitted;
 - (vii) to terminate this RFP process at any time, by notifying suppliers who submitted proposals, and, following termination, to negotiate with any supplier(s) on whatever terms PHARMAC thinks fit;
 - (viii) to re-advertise for proposals.
- (b) PHARMAC may consult or seek clinical advice from PTAC or its relevant sub-committee at any stage of the RFP process. PHARMAC will notify you if the clinical advice results in any changes to the terms of the RFP.
- (c) You must not initiate or engage in any communication with other suppliers in relation to the RFP, whether before or after submitting their proposal(s), until such time as the RFP process is completed.
- (d) You must not at any time initiate any communication with PHARMAC's directors or officers, the Ministry of Health, the Minister of Health or District Health Boards, with a view to influencing the outcome of this RFP process.
- (e) You must pay your own costs for preparing and submitting your proposal.
- (f) Proposals are submitted in reliance on your own knowledge, skill, and independent advice, and not in reliance on any representations made by PHARMAC.
- (g) Your submission of a proposal will be taken as acceptance of the terms contained in this RFP letter. PHARMAC may exclude your proposal if you do not comply with any of the terms contained in this RFP letter.
- (h) This is an RFP and not a tender. Your proposal is not an offer capable of being converted into a contract for the supply of the medicine for a rare disorder by PHARMAC's apparent acceptance and instead a separate agreement needs to be negotiated.

- (i) PHARMAC is not liable in any way whatsoever for any direct or indirect loss (including loss of profit), damage or cost of any kind incurred by you or any other person in relation to this RFP.
- (j) PHARMAC will consider your proposal and information exchanged between us in any negotiations relating to your proposal, excluding information already in the public domain, to be confidential to us and our employees, legal advisors and other consultants, the Ministry of Health and DHBs (**Confidential Information**). However, you acknowledge that it may be necessary or appropriate for PHARMAC to release Confidential Information:
 - (i) pursuant to the Official Information Act 1982; or
 - (ii) in the course of consultation on a provisional agreement entered into with a supplier; or
 - (iii) in publicly notifying any approval by the PHARMAC Board of that agreement; or
 - (iv) otherwise pursuant to PHARMAC's public law or any other legal obligations.

PHARMAC may consult with you before deciding whether to disclose Confidential Information for the purposes described in sub-clauses (i) to (iv) above. You acknowledge, however, that it is for PHARMAC to decide, in its absolute discretion, whether it is necessary or appropriate to disclose information for any of the above purposes, provided that PHARMAC shall act in good faith in disclosing any Confidential Information.

6. Anticipated timeline

- (a) Following receipt of proposals, PHARMAC anticipates:
 - (i) the Evaluation Committee evaluating proposals;
 - (ii) negotiating with submitter(s) of one or more preferred proposals;
 - (iii) consulting on a provisional agreement, or provisional agreements;
 - (iv) PHARMAC's Board, or the Board's delegate, considering this provisional agreement, or provisional agreements.

Schedule 3: Current listing and market information

PHARMAC staff have identified the following products that have been assessed (both by PTAC & PHARMAC) and prioritised for funding which might meet the proposed pre-requisites for the fund.

The information is indicative only and is estimated based on available international literature. PHARMAC makes no representation as to the accuracy of this information or as to the level of sales or likely sales of any of the medicines for rare diseases and, while PHARMAC has taken all reasonable care in preparing the information set out below, it accepts no liability for any errors or omissions in the information. PHARMAC is not obliged to notify you in the event of any change to the figures below.

Treatment	Indication	Prevalence (per 1 million [#])	NZ Patient Estimate	NPPA Applications
Agalsidase alfa	Fabry disease	2 to 7.5	9 to 34	8 (total for Fabry)
Agalsidase beta	Fabry disease	2 to 7.5	9 to 34	
Alglucosidase alfa	Pompe disease	2.0	9	7
Laronidase	MPS I	1.8 to 8.8	8 to 40	6
Idursulfase	MPS II	0.2 to 0.7	1 to 3	<5
Galsulfase	MPS VI	0.7 to 2.7	3 to 12	<5
Eculizumab	PNH	2.6	12-20	<5
Ivacaftor*	Cystic fibrosis, G551D	4.9	22	<5

[#]A prevalence of 1 in 50,000 is equivalent to 20 per 1 million.

*Ivacaftor has not completed PTAC review and prioritisation as PTAC deferred a recommendation

PHARMAC is also aware of a new product elosulfase alfa, which has not been assessed by PTAC, which was recently registered by the FDA in 2014:

Treatment	Indication	Prevalence (per 1 million)	Patient Estimate	NPPA Applications
Elosulfase alfa	MPS IVA	4	18	<5

PHARMAC considers that there are likely to be many other products that it has not been made aware of, or which it has not received funding applications for in the past, which might meet the proposed pre-requisites for the fund. Therefore the list of products above is not intended to restrict the products or bids we may receive.