PHARMAC
Pharmaceutical Management Agency

Level 9, 40 Mercer Street, Wellington 6011
PO Box 10-254, Wellington 6143, New Zealand
Phone 64-4-460-4990
Fax 64-4-460-4995
Information line **0800 66 00 50**
enquiry@pharmac.govt.nz
www.pharmac.govt.nz

7 August 2017 **(Amended 10 August 2017)**

Dear Supplier

REQUEST FOR PROPOSALS – SUPPLY OF A FIRST LINE ENZYME REPLACEMENT THERAPY FOR THE TREATMENT OF GAUCHER DISEASE

PHARMAC invites proposals for the supply of a First Line enzyme replacement therapy for the treatment of Gaucher disease.

This request for proposals (**RFP**) letter incorporates the following schedules:

- **Schedule 1** specifies the pharmaceutical for which PHARMAC is requesting proposals and sets out the background to the RFP and the types of proposals sought;
- **Schedule 2** describes the process that PHARMAC expects to follow in relation to the RFP;
- **Schedule 3** sets out information about the estimated size of the current subsidised market for the pharmaceuticals; and
- **Schedule 4** contains the RFP form in which you are to provide details of your proposal.

If you wish to submit a proposal, please submit it to PHARMAC via the Government Electronic Tenders Service (**GETS**) (www.gets.govt.nz) no later than **4.00 p.m**. on **Friday 8 September 2017**.

If you have any inquiries about this RFP you should submit them via GETS or alternatively contact Matthew Wolfenden, Procurement Manager, by email procurement@pharmac.govt.nz. Answers to questions will be provided through GETS. PHARMAC will also post any addenda through GETS. We encourage interested suppliers to register with GETS and subscribe to this RFP to be kept up to date.

We look forward to receiving your proposal.

Yours sincerely

Greg Williams
Acting, Director of Operations

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

1. Pharmaceutical

PHARMAC is interested in considering any proposal from suppliers of a First Line enzyme replacement therapy for the treatment of Gaucher disease.

2. Definitions

Enzyme replacement therapy (ERT) is a medical treatment which replaces an enzyme that is deficient or absent in the body.

First Line – where the pharmaceutical is funded for use as the first treatment for Gaucher disease.

3. Background to RFP

The background to this RFP is as follows:

Gaucher disease is a lipid storage disease characterised by the deposition of glucocerebroside in cells of the macrophage-monocyte system. The disorder results from the deficiency of a specific lysosomal hydrolase, glucocerebrosidase (also termed acid beta-glucosidase, glucosylceramidase). The disease is characterised by a continuum of phenotypes. The severity varies widely; some patients present in childhood with virtually all the complications of Gaucher disease, whereas others remain asymptomatic into the eighth decade of life.

Gaucher disease has traditionally been divided into 3 clinical subtypes (see below), delineated by the absence or presence of neurologic involvement and its progression. However, some cases do not fit precisely into one of these categories. All forms of Gaucher disease are autosomal recessive.

- Type 1 (**GD1**) The most common, affecting approximately 99% of patients and is non-neuronopathic and affects primarily adults;
- Type 2 (**GD2**) The most severe neuronopathic type and is generally fatal by 2 years of age; and
- Type 3 (**GD3**) Affects juveniles and is characterised by sub-acute neuropathological symptoms.

Treatment of Gaucher disease is tailored to the individual patient because of the variability in the manifestations, severity, and progression of the disease. The basic goals of treatment are elimination or improvement of symptoms, prevention of irreversible complications and improvement in overall health and quality of life. In children, an additional goal is the optimisation of growth. Patients are regularly monitored to determine disease progression and assess treatment success. Radiological imaging including MRI, serum chitotriosidase levels, visceral and haematological indices, growth, and pulmonary function are the main measures. More recently, a Gaucher specific biomarker, glucosylsphingosine, has been used to diagnosis and monitor Gaucher disease and this is now available in New Zealand.

Funding history

Currently ERT with imiglucerase (*Cerezyme* supplied by Sanofi Genzyme) is funded in New Zealand for eligible patients via application to the Gaucher Panel. Imiglucerase has been funded in New Zealand since July 1999. Prior to this, alglucerase *(Ceredase,* which

has been withdrawn from the market*)* was funded. The Gaucher Panel was established in 1997 to manage applications.

International recommended dosing of imiglucerase for GD1 is typically 30 to 60 iu/kg every two weeks (60 to 120 iu/kg/month). In New Zealand the funded doses of imiglucerase for patients with GD1 or GD3 who the Gaucher Panel considers meet the eligibility criteria is as follows:

- a maximum monthly dose of 15 iu/kg/month;
- a maximum monthly dose of 30 iu/kg/month for certain children; and

PHARMAC Board (or delegated authority) approval is required for doses greater than 30 iu/kg/month.

All patient clinical information and doses are reviewed annually, with some reviewed six monthly.

Dosage and administration

There are currently 20 patients receiving funded therapy for Gaucher disease in New Zealand. Eighteen patients have GD1, and two patients have GD3. Managing dosing and the number of vials used, for new and existing patients, is one of the key functions of the Gaucher Panel. It determines the number of vials for each patient based on weight to ensure appropriate dose rounding is applied to minimise wastage. Current dosing is detailed below for the 20 patients currently accessing treatment with imiglucerase (correct at June 2017):

Number of Patients	Dose (iu/kg/month)
11	15
6	30
3	30-60

In New Zealand, approximately half of the patients attend local hospital outpatient clinics to receive treatment with the other half of patients self-administering at home.

Current funding

The table below outlines the current Pharmaceutical Schedule listing of Imiglucerase (*Cerezyme*) in the *Gaucher's Disease* section of the *Metabolic Disorder Agents* sub-group of the *Alimentary Tract and Metabolism Therapeutic Group* Section B of the Pharmaceutical Schedule.

	Subsidy/ Price	Per	Fully Subsidised	Brand or Generic Manufacturer
IMIGLUCERASE Special Authority see SA0473 below – Retail pharmacy				
Inj 40 iu per ml, 200 iu vial ……	1,072.00	1	✓	**Cerezyme**
Inj 40 iu per ml, 400 iu vial …… S	~~2,114.00~~ **2,144.00**	1	✓	**Cerezyme**

SA0473 Special Authority for Subsidy:

- SA0473 – Imiglucerase (Cerezyme) therapy - initial application
- SA0473 – Imiglucerase (Cerezyme) therapy - entry and exit criteria
- SA0473 – Imiglucerase (Cerezyme) therapy - renewal application

The treatment is listed in Section B of the Pharmaceutical Schedule and is mainly administered by infusion in a hospital/outpatient setting on a fortnightly basis. Part III of Section H of the Pharmaceutical Schedule (the hospital medicines list) currently lists the Chemical and presentations without a brand or price.

Clinical advice

In February 2016, the Pharmacology and Therapeutics Advisory Committee (**PTAC**) reviewed an application for the funding of velaglucerase alpha for the treatment of Gaucher disease and recommended that PHARMAC run a Request for Proposals for a First Line enzyme replacement therapy for the treatment of Gaucher disease. The full minutes of the meeting are available on our website.

PHARMAC has carefully considered if it would be possible to change existing patients to a different Gaucher ERT treatment and has also sought advice from the Gaucher Panel. PHARMAC considers it would be appropriate to allow for a minimum six month transition period if a product switch eventuated from this RFP

Reasons for running the RFP

There is interest from new suppliers which has led to increased market interest which may allow PHARMAC to create savings, in turn allowing PHARMAC to increase the dosing of ERT for the small number of patients currently receiving treatment. Ultimately a standard Special Authority restriction may be possible, thus reducing PHARMAC's administration costs for managing the funding of treatments for this disease.

4. Types of proposals sought

PHARMAC is willing to consider the following types of proposals for First Line ERT for the treatment of Gaucher disease in New Zealand:

- Suppliers **must** submit a proposal for a First Line ERT for the treatment of Gaucher disease in New Zealand, under the current restrictions listed in Section B of the Pharmaceutical Schedule.
- Suppliers **may** also submit a proposal for a First Line ERT for the treatment of Gaucher disease in New Zealand, which allows higher IU/Kg dosing to be used. PHARMAC would likely seek clinical advice on any higher dosing proposals.
- Proposals **may** include a period of sole subsidised supply in the community (hereinafter referred to as "**Sole Supply**"), provided that the Sole Supply period does not extend beyond 30 June 2023;
- All proposals which would require a brand switch **must** include:
    - an option that permits imiglucerase to continue to be used as an alternative treatment for patients who have a clinical reason that would prevent them switching (i.e. allergic reaction to the new product or clinically unstable disease); and
    - a six month transition period between listing the new pharmaceutical and commencement of any Sole Supply arrangement.
- Proposals **may** include any of the following arrangements:

- rebates or other risk-sharing arrangements;
- a hard cap, provided that the hard cap would be for up to 5 years and then revert back to an average net cost per vial (with rebate if desired) thereafter;
- a soft cap/tiered cap, provided that a supplier also submits an alternative bid with a flat rebate structure.

PHARMAC would consider proposals where your product/s are yet to obtain all necessary Consents (where Consents means all consents, permits, licences and authorisations, whether statutory or otherwise, required for the supply of the pharmaceutical in New Zealand (including Ministry of Health market approval)). In those circumstances, you may be required to demonstrate your ability to obtain those Consents within a time frame acceptable to PHARMAC. For example, you may be required to demonstrate that you have the dossier for your product/s ready to submit to Medsafe within one month of such a request being made by PHARMAC.

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

- Proposals involving pharmaceuticals or related products other than ERT for the treatment of Gaucher disease.
    - For the avoidance of doubt, PHARMAC will not consider any proposals for Substrate Reduction Therapy Agents.
- Proposals that involve an end date for expenditure caps (other than as described for the hard cap above).
- Proposals that involve foreign currency exchange rate clauses or prices linked to any index.
- Two-part pricing arrangements, whereby PHARMAC may make an up-front payment (in addition to any ongoing subsidy) in return for the listing of a pharmaceutical on specific terms.

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

Samples

Suppliers should provide PHARMAC with samples and/or artwork of the products included in their proposal (and, if supply is intended to be of a product that differs from the samples, information about differences must be supplied) within 10 business days from the date specified in Schedule 2, clause 1 (b).

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 to PHARMAC **via the Government Electronic Tenders Service (GETS)** no later than **4.00 p.m.** (New Zealand time) on **Friday 8 September 2017**. Late proposals will only be considered at PHARMAC's discretion, taking into account the need for fairness to other suppliers and maintaining the integrity of the RFP process.

(c) You cannot withdraw your proposal, once submitted, while the RFP process is continuing.

(d) If you have any inquiries about this RFP you should submit them via GETS or alternatively contact Matthew Wolfenden, Procurement Manager, by email procurement@pharmac.govt.nz.

2. **Evaluation**

(a) Following the deadline for submitting proposals an Evaluation Committee comprising PHARMAC staff will evaluate each proposal to select its preferred proposal(s).

(b) The Evaluation Committee will evaluate proposals in light of PHARMAC's statutory objective which is "to secure for eligible people in need of pharmaceuticals, the best health outcomes that are reasonably achievable from pharmaceutical treatment and from within the amount of funding provided". In doing so the Evaluation Committee will be guided by the Factors for Consideration (**Factors**) that form part of PHARMAC's then current Operating Policies and Procedures (**OPPs**), as published on PHARMAC's website (www.pharmac.govt.nz), to the extent applicable. More information on the Factors can be found at www.pharmac.health.nz/factors-for-consideration.

(c) The requirement for PHARMAC to pursue its statutory objective means that particular emphasis will be given to those aspects of proposals which demonstrate "health outcomes", and those aspects of proposals which demonstrate the impact on the "funding provided" for pharmaceuticals. Those Factors which relate directly to these aspects will be given the greatest weight by the Evaluation Committee but all Factors are important.

(d) The information to be taken into account in applying the Factors by the Evaluation Committee will be at its discretion, however it will include:

(i) information provided by you in accordance with Schedule 4 of this RFP; and

(ii) any other information that the Evaluation Committee considers to be relevant having regard to probity principles.

(e) 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.

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

3. **PHARMAC may request further information**

(a) PHARMAC may request such further information as it considers necessary from or about you for the purposes of clarifying or evaluating your proposal.

(b) If PHARMAC requests further information from or about you, it is not obliged to request the same or any other information from or about any other party, provided that in PHARMAC's judgment this would not be unfair to any other party.

4. **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 either supplier's proposal would exclude acceptance of the other proposal.

(b) Negotiations will proceed on the basis that PHARMAC's standard terms and conditions for supply of pharmaceuticals, which are available on GETS, 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 with a different supplier(s).

5. **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 Factors in PHARMAC's then current OPPs.

(d) If the Board or its delegate does not approve the provisional agreement, then PHARMAC may initiate negotiations for a provisional agreement with any other supplier(s).

(e) The RFP process will be complete once PHARMAC has notified supplier(s) of either:

(i) the Board's or its delegate's decision to accept a negotiated agreement; or

(ii) the termination of the RFP process.

6. **Miscellaneous**

(a) PHARMAC reserves the right, having regard to probity principles:

(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; and

(viii) to readvertise 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) PHARMAC intends to include a provision, in any agreements that include Sole Supply arising from this RFP, that PHARMAC may elect at its sole discretion to extend the Sole Supply period beyond the initial Sole Supply period for any Pharmaceutical or Pharmaceuticals resulting from this RFP process, subject to the following:

(i) should PHARMAC elect to extend the initial Sole Supply period for a Pharmaceutical or Pharmaceuticals it shall do so by providing written notice to the supplier of the Pharmaceutical or Pharmaceuticals at least six months prior to the end date of the initial Sole Supply period;

(ii) should no written notice be provided by PHARMAC before the date being six months prior to the date of expiration of the initial Sole Supply period, no such extension shall occur; and

(iii) any extension of the Sole Supply period shall be for a maximum period of 24 months inclusive and shall take effect immediately following the end of the initial Sole Supply period.

(d) You must not initiate or engage in any communication with other suppliers in relation to the RFP, whether before or after you and/or they submit a proposal(s),

until such time as a provisional agreement is accepted by PHARMAC's Board or the Board's delegate.

(e) You must not at any time initiate any communication with PHARMAC, the Ministry of Health (including its operating unit Medsafe), the Minister of Health (or any Associate Ministers) or DHBs or advisors to PHARMAC with a view to influencing the outcome of this RFP process.

(f) You must pay your own costs for preparing and submitting your proposal.

(g) Proposals are submitted in reliance on your own knowledge, skill, and independent advice, and not in reliance on any representations made by PHARMAC.

(h) 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.

(i) 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 ERT by PHARMAC's apparent acceptance and instead a separate agreement needs to be negotiated.

(j) 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.

(k) 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.

7. Anticipated timetable

(a) Following receipt of proposals, PHARMAC anticipates:

(i) the Evaluation Committee evaluating proposals in **September/October 2017**;

(ii) negotiating with submitter(s) of one or more preferred proposals in **October 2017**;

(iii) if applicable, seeking advice from the Gaucher Panel and consulting on a provisional agreement by **November/December 2018**;

(iv) PHARMAC's Board or its delegate considering the provisional agreement in or after **February 2018**,

provided that the above time frames are only approximate and may be extended, without notice being required from PHARMAC, if any stages of the RFP process take longer than anticipated.

(b) Under this indicative timetable, the earliest that changes to the Pharmaceutical Schedule could be implemented is **1 March 2018**.

(c) Please note that if a proposal for Sole Supply is accepted, the date of implementation may be later to allow for an orderly transition to any Sole Supply arrangement. We would expect a six month transition period if there was a change of pharmaceutical.

8. **Governing Law**

This RFP is governed by New Zealand law, and the New Zealand courts have exclusive jurisdiction in all matters relating to this RFP.

Schedule 3: Current listing and market information

The following information relates to the estimated subsidised market size of imiglucerase.

The information is approximate and indicative only. PHARMAC makes no representation as to the accuracy of this information or as to the level of sales or likely sales of a First Line ERT for the treatment of Gaucher disease 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.

Imiglucerase Presentation	FYE 30 June 2015	FYE 30 June 2016
Inj 40 iu per ml, 200 iu vial	0 Units	15 units
	$0	$16,080
Inj 40 iu per ml, 400 iu vial	809 units	777 units
	$1,734,496	$1,665,888

Schedule 4: Proposal form

An electronic version of this form is available on PHARMAC's website at www.pharmac.govt.nz and on GETS (www.gets.govt.nz). You should expand the boxes as necessary.

[Supplier to insert date]

Director of Operations
PHARMAC
C/- Matthew Wolfenden

By electronic transfer using GETS (https://www.gets.govt.nz)

Dear Sir/Madam,

Proposal for the supply of a First Line enzyme replacement therapy for the treatment of Gaucher disease in New Zealand

In response to your request for proposals (**RFP**) dated 7 August 2017, we put forward the following proposal.

Set out below is further information in support of our proposal.

(a) Our contact details:

Name of supplier	
Contact person	
Address	
Phone	
Email address	

(b) Details of pharmaceutical presentation:

Chemical name	
Brand name	
Strength (e.g. 40 iu per ml, 400 iu vial)	
Form (e.g. injection)	
Pack size (e.g. 10)	
Packaging type (e.g. glass vial)	
Shelf life (including conditions)	
Indications	

(c) Key features of our proposal (please include details of any brand change and/or higher dosing implementation assistance):

(d) Information relating to pricing ($NZ, GST exclusive), including any related conditions or proposed terms affecting cost for PHARMAC (e.g. price in return for Sole Supply, allowing higher IU/Kg dosing, risk sharing mechanisms etc.):

(e) Evidence of market approval and any other required consents:

Date of market approval (please attach copy of Medsafe Gazette notice)	
OR Date of submission of dossier (please attach confirmation from Medsafe that dossier has been submitted)	
OR Expected date of dossier submission to Medsafe	
Insert any other consents required for pharmaceutical	

(f) Information about our ability to ensure the continuity of supply of the pharmaceutical (please include risk mitigation strategies):

(g) Confirmation that there are no intellectual property barriers (including patent barriers) to our supply of this product in New Zealand, with additional information if required:

(h) Information about our previous supply performance and relevant expertise:

(i) Proposals/suggestions (e.g. pricing, risk sharing arrangements etc) regarding the pharmaceutical not expressly identified in this RFP that we would like PHARMAC to consider as part of our proposal:

(j) Additional information that PHARMAC should consider when evaluating our proposal (Please include information you consider relevant under PHARMAC's Factors for Consideration decision making framework):
