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Guidelines

for Funding Applications to PHARMAC

Amended in 2015



PHARMAC
Pharmaceutical Management Agency

newzealand.govt.nz

Foreword

PHARMAC, the Pharmaceutical Management Agency, is primarily responsible for managing the funding of pharmaceuticals for New Zealanders, on behalf of the District Health Boards. PHARMAC's objective 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.

Each year, PHARMAC receives a large number of applications ("Applications") containing proposals to fund new pharmaceuticals or to widen access to pharmaceuticals that are already funded. As PHARMAC must work within a fixed budget, difficult choices need to be made about which of the proposals should be progressed to a funding decision at any given time. This involves assessing a large amount of often complex information to identify proposals that would provide the best health outcomes.

In deciding which proposals to fund, PHARMAC assesses each proposal against its nine Decision Criteria, which include consideration of health need and assessment of cost-effectiveness. All proposals are prioritised against all other funding options according to these criteria.

In this context of what we call 'relative assessment' (comparing one option against another), it is important that applicants provide good quality, complete and balanced Applications that follow these *Guidelines*.

Good quality Applications will include the following elements:

- critical appraisal of key clinical evidence;
- information relating to PHARMAC's nine Decision Criteria;
- complete market and epidemiological information;
- information on cost-effectiveness (based on PHARMAC's methodology for cost-utility analysis); and
- disclosure of information on all known ongoing trials and patents.

Good quality Applications will be clear, cite all sources, explain all assumptions, and include all information of material importance. A thorough yet succinct Application is likely to expedite PHARMAC's review, potentially enabling the proposal in the Application to be prioritised earlier.

Please note that the information requested in these *Guidelines* is not mandatory. The information we have requested is information that PHARMAC requires when assessing a proposal. The purpose of these *Guidelines* is to provide you with some guidance on how to compile an Application that is useful to us. We do not want to impose any unnecessary burden or create any barriers to submitting an Application. This is why we strongly recommend that you contact PHARMAC first before submitting an Application, as in certain circumstances, less information may be required.

If the information requested in these *Guidelines* is not provided, it is likely that PHARMAC will either contact you for it at some stage or undertake its own searches or analysis (which may result in time delays). Therefore, even though the information

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requested is not mandatory, there are advantages in providing thorough Applications following these *Guidelines*.

If the application is for the funding of a generic or biosimilar pharmaceutical, and the pharmaceutical (including the indication applied for) has previously been assessed by PHARMAC, less information may be required. Please refer to Section 8 of these *Guidelines* for further details.

Anyone can submit a funding Application to PHARMAC, however these guidelines may be of most use to pharmaceutical suppliers. We strongly encourage all applicants to contact PHARMAC (++64 (04) 460-4990 or 0800-66-00-50) before submitting an Application. You will be transferred to the relevant Therapeutic Group Manager who can answer any questions you may have and confirm what the appropriate procedure is and what information needs to be provided.

First Step

Please call PHARMAC (++64 (0)4 460-4990 or 0800-66-00-50) for all inquiries regarding Applications to fund new pharmaceuticals or to widen access to pharmaceuticals that are already funded. We will direct you to the relevant Therapeutic Group Manager.

Send all Funding Applications and supporting information to:

Manager, Pharmaceutical Funding
PHARMAC
Level 9
40 Mercer Street
PO Box 10-254
WELLINGTON 6143

Checklist for Funding Applications

The checklist below outlines the information required when submitting a Funding Application to PHARMAC for an amendment to the Pharmaceutical Schedule. If anything is unclear, please check the glossary in Appendix 1 and contact PHARMAC if you need further clarification.

CHECKLIST FOR FUNDING APPLICATIONS TO PHARMAC		
	Page	
CRITERIA		
Application is for an addition or amendment to the Pharmaceutical Schedule	9	Y/N
STRUCTURE OF APPLICATION		
Separate synopsis provided	16	Y/N
Spiral bound copies are clearly labelled and each section indexed	16	Y/N
Front cover states pharmaceutical name, brand name, date of Application, name of pharmaceutical supplier and name of applicant (including name, phone number and email address of contact person)	16	Y/N
Table of contents included	16	Y/N
Three hard copies provided and one electronic copy	16	Y/N
MAIN CONTENT		
Pharmacological information	19	Y/N
Proposed changes to the Pharmaceutical Schedule	20	Y/N
Epidemiological information	21	Y/N
Price information	22	Y/N
Market information	23	Y/N
Patent information	23	Y/N
Impact on health sector	24	Y/N
Search strategy used for identifying clinical trials	24	Y/N
Summary of the key randomised controlled trials (methods and outcomes)	27	Y/N
Assessment of the quality of the evidence	27	Y/N
Incidence and descriptions of adverse drug reactions	28	Y/N
Applicability of the evidence to the New Zealand health sector	28	Y/N
Summary of Application evaluated against PHARMAC's Nine Decision criteria	29	Y/N
MAIN ATTACHMENTS		
Medsafe approved datasheet	19	Y/N
New Zealand Medicines Assessment Advisory Committee (MAAC) response to the registration application	19	Y/N
Copies of all identified randomised controlled trials published in peer-reviewed journals, subdivided by grade of evidence and ordered by date of publication	25	Y/N
One complete hard copy of the clinical study report summaries from the pivotal trials	25	Y/N
Copies of all errata, editorials and journal correspondence relating to published trials	25	Y/N
Register of all ongoing trials on the pharmaceutical/ medical device	25	Y/N
Declaration that all known unpublished clinical trials have been disclosed	25	Y/N
One labelled sample of the pharmaceutical inside its packaging (unless Application is for widening of access)	31	Y/N

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CHECKLIST FOR FUNDING APPLICATIONS TO PHARMAC		
ADDITIONAL CONTENT		
Cost-utility analysis based on the methods outlined in the Prescription for Pharmacoeconomic Analysis (including all costs estimated in \$NZ)	33	Y/N
Additional information on health need and public health significance	35	Y/N
Additional information on health need by Maori and Pacific peoples	36	Y/N
ADDITIONAL ATTACHMENTS		
Copies of relevant EMEA European Public Assessment Reports (EPARs) and FDA Medical reviews	32	Y/N
Review articles and published critiques	32	Y/N
International guidance and recommendations	32	Y/N
Published cost-utility analyses	32	Y/N
Expert opinion and consensus reports from expert panels	32	Y/N
Where a cost-utility analysis is included, an electronic copy of the TreeAge™ model or Excel™ spreadsheet	33	Y/N
REAPPLICATIONS		
The Reapplication addresses all of the issues/concerns raised when the original Application was assessed	38	Y/N
List of all new information provided, along with copies of the new information	38	Y/N
APPLICATIONS FOR THE FUNDING OF GENERIC OR BIOSIMILAR PHARMACEUTICALS		
Registration and indication details	39	Y/N
Pharmaceutical information (including one labelled sample)	39	Y/N
Price information	40	Y/N
Market information	40	Y/N
Patent information	40	Y/N
Lead times	40	Y/N
APPLICATIONS FOR THE FUNDING OF BLOOD GLUCOSE METER SYSTEMS		
International information	41	Y/N
Product information	41	Y/N
Price information	42	Y/N
Lead times	42	Y/N
Product testing	42	Y/N

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1. Background

1.1 What is PHARMAC?

PHARMAC, the Pharmaceutical Management Agency, is a Crown Entity that is directly accountable to the Minister of Health. Our functions are set out in section 48 of the New Zealand Public Health and Disability Act 2000 (NZPHD Act).

One of PHARMAC's functions is to manage the Pharmaceutical Schedule, which is the list of pharmaceuticals that are publicly funded. Thus, we may refer to a funded pharmaceutical as being "listed," or to funding applications as containing proposals to "list" a pharmaceutical or to "make amendments to the Pharmaceutical Schedule".

We also negotiate national contracts for pharmaceuticals and products used by District Health Board (DHB) hospitals and these are also listed in the Pharmaceutical Schedule (Part II of Section H, also known as the Hospital Medicines List or HML).

PHARMAC's statutory objective 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." Section 47(a) of the NZPHD Act

Further information about PHARMAC can be found at:
<http://www.pharmac.health.nz/>.

1.2 Purpose of the Funding Application Guidelines

The purpose of these *Guidelines* is to provide guidance on how to prepare applications to PHARMAC for proposed amendments to the Pharmaceutical Schedule ("Applications").

These *Guidelines* are subject to the policies and procedures set out in PHARMAC's Operating Policies and Procedures:

<http://www.pharmac.health.nz/about/operating-policies-and-procedures>

This document is intended to assist anyone wanting to make an Application to PHARMAC for a proposed amendment to the Pharmaceutical Schedule. See section 2.2 of this document for further information on the use of these *Guidelines*.

2. The Application Process

PHARMAC has an established process for deciding which pharmaceuticals, and which of their possible uses (indications), to fund. The decision-making process described in this document is the process that PHARMAC generally follows with an Application for a proposed change to the Pharmaceutical Schedule. PHARMAC may occasionally adopt a different process, or vary the process (for example, we decide whether or not it is appropriate to undertake consultation on a case-by-case basis).

2.1 Who can initiate an Application?

There are no restrictions on who can make an Application. Applications are usually made by pharmaceutical suppliers; however, clinicians, interest groups, PHARMAC committees and consumers (patients) may also make Applications.

Before making an Application for a proposed amendment to the Pharmaceutical Schedule, please call PHARMAC on +64 (0)4 460-4990 or 0800-66-00-50. We will direct you to the relevant Therapeutic Group Manager who will discuss the nature of the proposed amendment with you. They will confirm what the appropriate procedure is, and what information needs to be provided to PHARMAC.

2.2 When do these Guidelines apply?

Proposed amendments to the Pharmaceutical Schedule where these *Guidelines* apply include Applications for:

- (i) funding of new pharmaceuticals for use in the community;
- (ii) funding of new hospital pharmaceuticals;
- (iii) changing funded access to an already listed pharmaceutical (e.g. for new uses or patient groups);
- (iv) funding generic or biosimilar pharmaceuticals where an application to fund the pharmaceutical has not previously been considered by PHARMAC;
- (v) funding new formulations or strengths of already funded pharmaceuticals; and
- (vi) funding combination products (products that consist of two or more pharmaceuticals).

Funding Applications relating to a generic or biosimilar pharmaceutical where funding of the pharmaceutical has previously been considered by PHARMAC should follow Section 8 of these *Guidelines* only.

These *Guidelines* do not apply to funding of pharmaceuticals for individual patients. More information for the funding of individual through the Named Patient Pharmaceutical Assessment process can be found here: <http://www.pharmac.health.nz/tools-resources/forms/named-patient-pharmaceutical-assessment-nppa-forms>.

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Pharmacology and Therapeutics Advisory Committee (PTAC) or, in some cases, one of the specialist PTAC Subcommittees.

PHARMAC, in consultation with the PTAC Chair, may consider:

- (i) whether a particular Application should be referred directly to PTAC for advice; or
- (ii) whether a particular Application should be referred directly to the relevant Subcommittee (i.e. prior to its consideration by PTAC); or
- (iii) whether it wishes to invite relevant medical groups and other interested parties to comment on the pharmaceutical that is the subject of the Application before the Application is considered by PTAC or a Subcommittee. Where we seek comments, the main objective will be to enable interested parties to outline specific issues relating to the pharmaceutical (in relation to PHARMAC's decision criteria) early in the PHARMAC decision making process. These comments will then be considered by PTAC or a Subcommittee when it considers the Application.

The decision to submit an Application to PTAC (including the decision about which PTAC meeting to send it to) following receipt of the Application is at the discretion of PHARMAC in consultation with the PTAC Chair. If PHARMAC decides not to submit the Application to PTAC, PHARMAC will inform the applicant of the reasons for this.

If an Application is submitted for consideration at a particular PTAC or Subcommittee meeting, PHARMAC staff will usually draft a cover paper for the Application, which generally includes (but is not limited to) the following information:

- summary of the proposal (the pharmaceutical, indications, pharmaceutical supplier, proposed subsidy, etc.);
- questions to PTAC and/or the Subcommittee;
- previous PTAC and/or Subcommittee minutes (if relevant);
- brief description of the disease and current treatment in New Zealand;
- brief description of the pharmaceutical under consideration (including clinical evidence and any proposed restrictions or changes to access);
- international prices of the pharmaceutical;
- estimated cost of funding;
- cost-effectiveness (if available and relevant); and
- PHARMAC's decision criteria and Government priorities for funding.

2.4.1 PTAC Review of Applications

PTAC is PHARMAC's primary clinical advisory committee. Its role is to provide objective advice to PHARMAC on pharmaceuticals and their benefits. PTAC's members are appointed by the Director-General of Health in consultation with the PHARMAC Board. PTAC comprises senior health practitioners with expertise in critical appraisal and broad experience and knowledge of pharmaceuticals and their therapeutic uses. There are also a number of PTAC Subcommittees, made up of

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experts in specialist clinical fields such as cardiology and oncology. PHARMAC and/or PTAC often seek advice from a specialist PTAC Subcommittees.

When considering an Application, PTAC will review and critically appraise the clinical evidence. It uses the same decision criteria as PHARMAC when evaluating Applications. PTAC makes recommendations to PHARMAC regarding amendments to the Pharmaceutical Schedule and assigns priority ratings to these recommendations (typically high, medium or low). PTAC may also recommend that an Application be cost-neutral, declined or deferred, giving reasons for the deferral, such as supply of further information. When making recommendations to PHARMAC, PTAC indicates which Decision Criteria it has given particular weight to. These recommendations are taken into account when PHARMAC sets its funding priorities. Generally, if a proposal in an Application is given a high PTAC priority and the proposed amendment to the Pharmaceutical Schedule is relatively cost-effective, it may be progressed sooner than a proposal that has been given a low PTAC priority or one that is not as cost-effective. A positive recommendation by PTAC and/or its Subcommittees, however, is no guarantee of funding as the role of PTAC and PTAC Subcommittees is to advise PHARMAC on matters referred to them. PHARMAC is not bound to follow the recommendations made.

If PTAC considers that further specialist advice is needed prior to making a recommendation to PHARMAC, the Application may be referred to a PTAC Subcommittee. Applications may also be referred to PTAC Subcommittees for advice on developing or refining access criteria. If PTAC considers that further information is required from the applicant, this will be referred back to the applicant.

A copy of the relevant part of the PTAC/subcommittee minute is sent to the applicant. The applicant then has 10 working days to review the minute and to make a request for the withholding of all or part of the minute from public release. When we consider requests for withholding part or all of the PTAC/Subcommittee minutes, PHARMAC is guided by the grounds for withholding information specified in the Official Information Act. In some instances where the Application has not been made by the pharmaceutical supplier, the minute may be sent to the relevant pharmaceutical supplier as well as the applicant for review and feedback. This usually occurs if we consider that there is a possibility that the supplier may object to the release of part or all of the minute. Following this review, the minutes may be published on the PHARMAC website.

Further details about PTAC and PTAC Subcommittees can be found in the PTAC Terms of Reference, which are available on the PHARMAC website <http://www.pharmac.health.nz/about/committees/ptac/> or by contacting PHARMAC on +64 (0)4 460-4990.

2.4.2 Economic Assessment

PHARMAC generally will undertake or review two forms of analysis on a proposal:

- (i) a cost-utility analysis (CUA); and
- (ii) a budget-impact analysis (BIA).

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PHARMAC estimates the budgetary impact of the proposed change to the Pharmaceutical Schedule, usually over a period of five years (discounted at 8%)¹. In some cases a longer time horizon is required.

The methods PHARMAC uses when undertaking CUA are outlined in PHARMAC's Prescription for Pharmacoeconomic Analysis (PFPA) – <http://www.pharmac.health.nz/assets/pfpa-final.pdf>

We encourage applicants to provide a CUA when submitting an Application. Providing good quality analysis, following the methods outlined in the PFPA, may help the review and information acquisition process, enabling your Application to be prioritised earlier.

When PHARMAC receives a CUA from an applicant, our health economists review it, and amend it if required. In order to be able to review CUAs more efficiently, applicants should provide a copy of the TreeAge model and/or Microsoft Excel spreadsheet. If PHARMAC has made amendments to the analysis, PTAC will usually be supplied with a copy of the applicant's CUA and PHARMAC's amended CUA, with the differences between them clearly explained.

If an economic analysis has not been provided in the Application to illustrate the cost-effectiveness of the proposed amendment to the Pharmaceutical Schedule, PHARMAC will undertake a CUA. The stage at which this occurs depends on availability of health economist resources and its PTAC priority.

Very few Applications receive a detailed CUA assessment, as these take around 2–6 months to complete and, tend therefore to be too slow and resource-intensive for a purchasing environment. The process is usually iterative, meaning that rapid assessments are conducted first; then preliminary assessments; then if this is insufficient to inform a recommendation, an indicative or detailed analysis is undertaken. The level of analysis undertaken depends on the factors outlined in Table 1 below.

Table 1: Determinants of Level of Analysis Undertaken by PHARMAC

Determinants of level of analysis	Details
Timeframes	In some cases a CUA result may be required within a week; hence a more detailed analysis cannot be undertaken.
Impact on pharmaceutical budget	A high expenditure pharmaceutical is more likely to require a detailed CUA, especially if the pharmaceutical is highly effective.
Reliability of results	If the results of a CUA are very sensitive to key assumptions a higher level of analysis may be required.
Extent of information available for analysis	Pharmaceuticals for rare conditions are more likely to undergo rapid analysis due to unavailability of data.
Impact of CUA on funding decision	In some cases the pharmaceutical may be funded based on other decision criteria, hence a detailed analysis may not be required.

¹ Note that the discount rate used by BIA differs from the rate used for CUA (the (nominal) discount rate for BIA is 8%, and the (real) discount rate for CUA is 3.5% for both costs and benefits). The rationale for using these rates is outlined in the Prescription for Pharmacoeconomic Analysis (PFPA) - <http://www.pharmac.health.nz/assets/pfpa-final.pdf>

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Determinants of level of analysis	Details
Availability of health economist resources	Given limited health economist resources, it may not be cost-effective to undertake a detailed analysis when a number of other CUAs are also required.

Most CUAs are written up as 'Technology Assessment Reports' following a template. CUAs are then peer-reviewed internally and examination of the economic methodology is conducted. Analyses may also be reviewed by PTAC or the relevant specialist PTAC Subcommittee, or by other relevant external experts.

PHARMAC has no threshold below which a proposed amendment to the Pharmaceutical Schedule is considered "cost-effective". The main reason for this is that cost effectiveness is only one of the Decision Criteria we use. One Application may be more cost-effective than another but rate poorly on other Decision Criteria and, therefore, may not be progressed since, on 'successfulness grounds', it will not be considered cost effective. Another reason for not having a threshold value is that spending on community pharmaceuticals is required to be kept within a fixed budget. Given this constraint, and all other things being equal, what is and is not considered "cost-effective" will vary with the amount of funding available (not just in terms of the total budget each year, but the available budget at any point in time). An Application to fund a pharmaceutical can, therefore, only be considered "cost-effective" in comparison with other Applications under consideration by PHARMAC at any one particular time.

2.4.3 Prioritisation

Once full information on an Application is available (including PTAC priority and cost-effectiveness where necessary), it is compiled and considered by PHARMAC according to our nine Decision Criteria (outlined below).

<p>PHARMAC's Decision Criteria are:</p> <ol style="list-style-type: none">1. The health needs of all eligible people within New Zealand.2. The particular needs of Māori and Pacific peoples.3. The availability and suitability of existing medicines, therapeutic medical devices and related products and related things.4. The clinical benefits and risks of pharmaceuticals.5. The cost-effectiveness of meeting health needs by funding pharmaceuticals, rather than by using other publicly-funded health and disability support services.6. The budgetary impact (in terms of the pharmaceutical budget and the Government's overall health budget) of any changes to the Pharmaceutical Schedule.7. The direct cost to health service users.8. The Government's priorities for health funding, as set out in any objectives notified by the Crown to PHARMAC, in PHARMAC's Funding Agreement, or elsewhere.9. Any other criteria that PHARMAC thinks are relevant. PHARMAC will carry out the necessary consultation whenever we intend to take any 'other criteria' into account.
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All Applications are prioritised against other funding options (either listing of new pharmaceuticals or widening access to pharmaceuticals that are already listed), whether received via Application or via PHARMAC initiated proposals. The overall aim is to identify potential amendments to the Pharmaceutical Schedule that would provide the greatest possible health benefits and help us to meet our Statutory

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Objective. PHARMAC conducts regular prioritisation reviews of all outstanding Applications.

2.4.4 Negotiations

Therapeutic Group Managers are responsible for negotiating listing and supply agreements with pharmaceutical supplier(s), where these are relevant to a proposed change to the Pharmaceutical Schedule. This commercial activity may include: price negotiations; Special Authority or other targeting criteria; expenditure caps; rebates on the pharmaceutical price; and/or multi-product agreements. Negotiation outcomes may lead to re-prioritisation of an Application.

2.4.5 Decision

Section 49(a) of the NZPHD Act requires that PHARMAC must, when it considers it appropriate to do so, consult on matters that relate to the management of pharmaceutical expenditure with any sections of the public, groups or individuals that may be affected by decisions on those matters.

Prior to PHARMAC making a decision on a proposed change to the Pharmaceutical Schedule, we will, when we consider it appropriate, consult with people that may be affected by the proposed change (which may, according to the circumstances, include suppliers, PTAC and PTAC Subcommittees, health professionals, patients groups, Maori, Pacific peoples and other groups). Consultation responses are considered by PHARMAC with an open mind and, if appropriate, the proposal may be amended.

Decisions regarding any amendments to the Pharmaceutical Schedule are made by the PHARMAC Board, PHARMAC's Chief Executive acting under Delegated Authority, or PHARMAC's Director of Operations acting under delegated authority.

3. Structure of Applications

Applications to PHARMAC for the listing of new pharmaceuticals, new indications and/or new formulations should be separated into the following three sections:

- (i) synopsis;
- (ii) the main body of the Application; and
- (iii) supporting information.

Hard copies of the Applications should be provided in separate spiral bound volumes. It is particularly important that copies of the synopsis are provided separately from the rest of the Application.

Each binder should be clearly labelled and the Application indexed so that information can be easily located. Please provide a table of contents in each binder. It is also useful if the Application has labelled tabs for each of the sections it contains (i.e. pharmacology, epidemiology, etc.).

The front cover of the Application should include the following information:

- (i) pharmaceutical, brand name and indication;
- (ii) date of the Application; and
- (iii) the name of the pharmaceutical supplier and (if different) the name of the applicant (including name, phone number and email address of contact person).

The Application must include all relevant information known to the applicant, including data that is contrary to, or does not necessarily support, the case presented in the Application. Any information required for the Application (as outlined below) that is not available or is otherwise not supplied, should be stated explicitly under the relevant heading(s). Explain all sources and assumptions. If PHARMAC is not provided with information it considers necessary to assess the Application, this is likely to result in delays. Please be clear and succinct, yet thorough.

Applications must also include one labelled sample of the pharmaceutical inside its New Zealand packaging, as appropriate. Sometimes more samples may be required. A sample is not required with Applications for widening access to treatments already listed on the Pharmaceutical Schedule. Please refer to Section 5.10 for further details on the provision of samples.

3.1 Submitting an Application

Applicants must initially provide three hard copies and one electronic copy of the Application to PHARMAC. PHARMAC will undertake an initial screening of the Application (as outlined in section 2.4), usually within ten working days of receiving it, to ensure that it is complete and includes the key information that PHARMAC requires in order to assess the proposal. If the Application is incomplete or further clarification is required, PHARMAC may contact the applicant and may defer consideration of the Application until any outstanding issues are resolved.

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If an Application is approved for consideration by PTAC, PHARMAC will notify the applicant. Following notification, the applicant must provide fifteen copies of the Application.

Applicants must also supply one additional electronic copy of the entire Application. If an economic analysis is included in the Application, please also provide a copy of the analysis along with the TreeAge model and/or Excel spreadsheet as outlined in Section 2.4.2.

If you have any questions, please contact the relevant Therapeutic Group Manager at PHARMAC.

4. Information Required in the Synopsis to an Application

Sections 4 and 5 outline the information required for Applications to PHARMAC for new pharmaceuticals, new indications (expanded access) and new formulations.

Applications for generic or biosimilar pharmaceuticals (where the pharmaceutical has previously been considered by PHARMAC) should refer to Section 8.

Please refer to Section 7 of these *Guidelines* for details on the format and content required for reapplications.

4.1 Synopsis

The synopsis should include a high-level summary of key aspects and issues presented in the Application. This should generally not be more than 10 pages long and should include the following information:

- (i) official or approved names of the pharmaceutical;
- (ii) form(s), strength(s), pack size(s);
- (iii) registered indication(s);
- (iv) proposed restriction(s) for listing;
- (v) recommended course of treatment;
- (vi) main comparator(s) and the main expected changes in the clinical management algorithm;
- (vii) numbers of patients treated (restricted and unrestricted listing);
- (viii) proposed price;
- (ix) net cost of the proposed drug each year over five years;
- (x) the number and date of expiry for all relevant New Zealand patents (both granted and pending);
- (xi) cost per patient per course (for acute therapy) or the cost per patient per year (for chronic therapy);
- (xii) any wider health sector resources affected by the listing of the proposed drug;
- (xiii) main results of the clinical evaluation in terms of comparative effectiveness and comparative toxicity, including key data sources; and
- (xiv) if available, a summary of the cost-utility analysis, including the main sources of uncertainty in the structure and variables in the economic evaluation and the results of associated sensitivity analyses.

The synopsis will be used as a general information guide for PHARMAC and will be provided to PTAC members.

5. Information Required in the Application

5.1 Pharmacological Information

Please provide the following information:

- (i) official or approved names of the pharmaceutical;
- (ii) pharmaceutical form (e.g. ampoule, vial, sustained-release tablet);
- (iii) pharmaceutical strength;
- (iv) arranged pack sizes (in the case of a preparation such as an aerosol, state the number of doses available from the container);
- (v) principal pharmacological action of the pharmaceutical;
- (vi) indications registered for use in New Zealand – attach the Medsafe approved data sheet and the New Zealand Medicines Assessment Advisory Committee (MAAC) response to the registration application;
- (vii) where known, names of OECD countries where registration has been approved or declined;
- (viii) where known, names of OECD countries where an application for funding has been approved or declined;
- (ix) other presentations or formulations of the pharmaceutical that have been submitted for approval, or are already approved, in other OECD countries;
- (x) recommended dosages for each of the indications provided in connection with (vi) above. In the case of a pharmaceutical that is not used for chronic therapy, provide information on the average length of a treatment course and anticipated frequency of repeat courses of treatment;
- (xi) any contra-indications and drug interactions. Include information on any necessary dosage adjustments and cautions required when using the pharmaceutical in conjunction with other pharmaceuticals;
- (xii) common adverse effects (including frequency);
- (xiii) serious adverse effects; and
- (xiv) a summary statement of the main therapeutic claims for the pharmaceutical and its proposed use.

5.2 Proposed Changes to the Pharmaceutical Schedule

Please provide the following information:

- (i) the therapeutic group and/or subgroup where the applicant considers the pharmaceutical should be listed on the Pharmaceutical Schedule;
- (ii) details of any proposed restrictions to access (e.g. Special Authority criteria, endorsement criteria or hospital Restrictions);
- (iii) details of the comparator treatments available in New Zealand (funded and unfunded);
- (iv) present the current clinical treatment algorithm (or pathway) and outline the main expected changes in the treatment algorithm;
- (v) describe how the pharmaceutical compares clinically with pharmaceuticals already listed on the Pharmaceutical Schedule, including:
 - what (if any) advantages the pharmaceutical offers over existing listed pharmaceuticals in terms of efficacy and/or side effects:
 - whether the pharmaceutical is equivalent to existing listed pharmaceuticals
 - whether the pharmaceutical is more effective than existing listed pharmaceuticals
 - whether the pharmaceutical has a similar efficacy to existing listed pharmaceuticals but has fewer side effects
 - whether the pharmaceutical is associated with similar, greater or fewer side effects and/or toxicity than existing listed pharmaceuticals
 - whether the pharmaceutical offers greater compliance (e.g. once daily dosing) than existing listed pharmaceuticals (include evidence supporting this claim);
- (vi) if available, include dose equivalencies with comparator pharmaceuticals (whether listed or not) and justify these. State whether dose equivalencies were derived from direct or indirect comparisons;
- (vii) whether the pharmaceutical has a longer shelf life than existing listed pharmaceuticals, or other points of difference; and
- (viii) other pharmaceuticals, medical devices, related products or things, if any, likely to be prescribed for use in conjunction with the pharmaceutical as part of a course of treatment (whether listed or not). Include pharmaceuticals that may be used to manage any side effects.

5.3 Epidemiological Information

For each requested indication(s), please provide estimates for the first five years of the proposal (shown on a year-by-year basis) of:

- (i) the number of people in New Zealand with the particular condition(s);
- (ii) where available, the number of Māori people in New Zealand with the particular condition(s), along with their age-standardised prevalence rate per 1000 population;
- (iii) where available, the number of Pacific people in New Zealand with the particular condition(s), along with their age-standardised prevalence rate per 1000 population;
- (iv) where available, the number of non Māori / non Pacific people in New Zealand with the particular condition(s), along with their age-standardised prevalence rate per 1000 population;
- (v) the number of additional people in New Zealand likely to be prescribed the pharmaceutical under the proposal; and
- (vi) a breakdown of the number of people in New Zealand treated for the condition, including:
 - those who can be successfully treated by the pharmaceutical only;
 - those who can be treated by both the pharmaceutical and other pharmaceuticals (whether listed or not) that treat the same condition;
 - those who can be treated by only other pharmaceuticals (whether listed or not);
 - those who can be treated, completely or partially, by other therapies.

In all cases where estimates and assumptions are made or used, please clearly and explicitly state what underlies them, including sources. These may be indicated in ranges (i.e. the minimum and maximum plausible range of values).

Further information on deriving numbers of Maori and Pacific people with the particular condition(s) can be found in Section 6.6.

When estimating the likely number of patients eligible under access scenarios, please use an epidemiological approach. This means the prevalence and/or incidence of the condition to be treated; current patterns of how the pharmaceutical is used across different disease/indications; patterns over time in usage across diseases/indications; and patterns seen in other pharmaceutical markets internationally with the introduction of the new pharmaceuticals.

Please cite data sources, and clearly and explicitly state the bases of the assumptions made for the estimated numbers, including the quality of the data and relevance to the New Zealand setting. Estimates and assumptions can be indicated in ranges or similar.

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The table below provides a suggested format for presenting this information.

Assumption/ parameter	Central estimate	Type of estimator used (mean, median, mode, etc.)	Variation (as a range, percentile, standard deviations, 95% confidence interval, etc.)	Source (e.g. publication, reference from the literature - state; expert estimate (who/credentials)	Rationale for using this/these particular source/s

As with evidence of effectiveness (Section 5.8), all prevalence estimates and assumptions should be obtained systematically. Please supply details of the search strategies used to identify these (the medium used; databases searched; time period when undertaken; search strategy and keywords/MeSH headings used). Also specify the pre-defined inclusion and exclusion criteria for selecting the relevant epidemiological data used for estimates and assumptions.

Prevalence and outcomes data may be located using relevant on-line Medline searches (<http://www.ncbi.nlm.nih.gov/pubmed>) using search terms that incorporate [the disease/indication], (epidemiology OR prevalence OR risk factors OR natural history OR prognosis OR outcomes OR survival), and specific outcomes relevant to the disease/indication. Local epidemiology/outcomes data can be located more specifically by including the terms (Austral* OR Zealand OR NZ OR Aotearoa) in the search. If you are unsure about how to do such searches (including search strategy and keywords/MeSH headings), you may wish to discuss with a medical librarian or PHARMAC.

You may find useful sources of New Zealand mortality and morbidity data in cancer registry data, hospitalisation episode data, mental health episode data, and mortality data. These are available from Ministry of Health found at <http://www.health.govt.nz/nz-health-statistics>

5.4 Price Information

Please provide the following price information:

- (i) the supplier's selling price in \$NZ (ex-manufacturer, GST exclusive);
- (ii) the supplier's selling prices to wholesalers in other OECD countries where the pharmaceutical is marketed (in local currencies (excluding local taxes) and New Zealand dollar equivalents – please specify the exchange rates used); and
- (iii) alternative pricing proposals (e.g., possible price/volume trade-offs).

5.5 Market Information

Please provide the following market information:

- (i) estimated average daily dose (ADD) information for New Zealand (and other markets where possible) and estimated average daily cost (ADC) of treatment for New Zealand;
- (ii) expected sales (dollars and volume) for the first five years of listing, to be shown on a year-by-year basis with anticipated market segments and projected market shares; and
- (iii) estimate the change in the extent of use of other pharmaceuticals.

The ADD, ADC and expected sales information referred to in (i) and (ii) above should be supported by data from major OECD markets and other markets where the pharmaceutical is available (i.e. therapeutic indication(s) and use, ADD information, ADC of treatment, and sales). These data should cover the time period from product launch within each market to the date of the Application, on a year-by-year basis.

5.6 Patent Information

Please provide the information set out below on each patent (granted and pending) that has claims relating to the pharmaceutical that is the subject of the Application, and with respect to which a claim of patent infringement could reasonably be asserted if a person, not licensed by the owner of the patent, distributed or sold the pharmaceutical in New Zealand.

This information is required to help inform PHARMAC's assessment of the budget impact associated with the Application.

Please submit the following information for each pharmaceutical substance (ingredient) patent and finished pharmaceutical product (formulation and composition) patent and method of use and "Swiss-type" claim patents:

- (i) patent number and the date on which the patent will (or would, if granted) expire;
- (ii) type of patent (i.e. drug substance, finished drug product, method of use or "Swiss-type" claim);
- (iii) name of the patent owner;
- (iv) if you are not the patent owner, details of your right to sell or distribute the pharmaceutical in New Zealand; and
- (v) if you or the patent owner do not reside or have a place of business within New Zealand, the name of your representative or the representative of the patent owner who resides or maintains a place of business within New Zealand and who is authorised to receive notices related to the patent.

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PHARMAC does not require information on manufacturing process patents.

If a patent is issued after the Application is made to PHARMAC but before funding is approved, please submit the required patent information in an amendment to the Application within 30 days of the date of issuance of the patent.

If you believe that there are no relevant patents with respect to which a claim of patent infringement could reasonably be asserted if a person, not licensed by the owner of the patent, distributed or sold the pharmaceutical in New Zealand, please specify this in your Application.

PHARMAC recognises that some applicants, particularly those that are not pharmaceutical suppliers, may not have access to this information. If this is the case, please specify it in your Application. PHARMAC may subsequently seek such information from the relevant pharmaceutical supplier.

5.7 Impact on Health Sector

Please provide information on the following:

- (i) hospital outpatient or community-based services required for administration of the pharmaceutical (e.g. nurse and specialist time required for infusions);
- (ii) laboratory and diagnostic tests;
- (iii) inpatient hospitalisation;
- (iv) emergency department visits;
- (v) specialist visits and primary care services; and
- (vi) community-based services (e.g. nurse home visits, residential care (specify), home help, hospice care).

If possible, please quantify this impact (refer to Section 7 of the PFFA for details on how to estimate these costs).

5.8 Evidence of Effectiveness and Safety

Please use key clinical data sources when estimating relative treatment effects, including published randomised controlled trials (RCTs) and meta-analyses. Other possible sources include observational studies, unpublished trial data, expert opinion, and case reports.

5.8.1 Search Strategy

All evidence should be obtained systematically. Describe the search strategy used to identify clinical studies, including:

- (i) who carried out the search? Using which medium? What databases were searched?
- (ii) over what time period was the search undertaken?
- (iii) what search strategy and keywords/MeSH headings were used?

Input / Output	Recommendation
Sensitivity Analysis	Sensitivity analysis should include univariate (simple) analysis, multivariate analysis and extremes (scenario) analysis.

6.3 Health Need and Public Health Significance

PHARMAC encourages applicants to provide further information on health needs and public health significance (beyond the information provided in Section 5.3) when submitting a funding Application. This information relates to PHARMAC's Decision Criterion 1, and may include:

- (i) Description of the burden of the disease or condition (impact of the disease or condition on the individual and the community), including:
 - the annual incidence of the disease/condition in New Zealand (number of new cases each year);
 - the morbidity associated with the condition (e.g. annual number of hospitalisations);
 - the premature mortality associated with condition in New Zealand (e.g. annual number of deaths; number of potential years of life lost before age 80 (PYLL(80))²);
 - the average disability-adjusted life years (DALYs) lost by an individual patient due to the disease(s)³;
 - the population loss of disability-adjusted life years (DALY loss) for the disease(s); and
 - the population DALY loss attributable to the disease as a percent of all diseases (where in 1996 there were 543,000 total DALY lost from all diseases⁴).

- (ii) Description of the estimated uptake rate of the pharmaceutical (i.e. the number of people likely to be prescribed the pharmaceutical, divided by number of people with the particular disease or condition who should benefit from the pharmaceutical). This should use both the epidemiological data above and in section 5.3 and the estimates of likely usage from section 5.3.

² PYLL80 = sum across all individuals of (80 years minus age at death for an individual)

³ Where an individual's normal life expectancy minus their loss of disability-adjusted life years (DALY loss) from their disease/condition equals their disability-adjusted life expectancy (DALE)

⁴ The Burden of Disease and Injury in New Zealand. Public Health Intelligence Occasional Bulletin No. 1. Wellington: Ministry of Health, 2001.

[http://www.moh.govt.nz/notebook/nbbooks.nsf/0/E916C53D599AE126CC2569F0007AAA23/\\$file/BurdenofDisease.pdf](http://www.moh.govt.nz/notebook/nbbooks.nsf/0/E916C53D599AE126CC2569F0007AAA23/$file/BurdenofDisease.pdf)

6.4 Health Need of Māori and Pacific peoples

The health needs of Māori and Pacific peoples is something PHARMAC is particularly keen to improve. This is reflected in our Decision Criteria (Criterion 2). PHARMAC encourages applicants, where possible, to provide information on health needs and public health significance specific to Māori and Pacific peoples when submitting a funding Application. This information should include the following:

- (i) A description of the availability and quality of data indicating the extent of disparity between Māori, Pacific peoples and non-Māori/non Pacific people. This may include further data on disease prevalence, incidence, notifications of new cases, public hospital hospitalisations, and/or deaths.
- (ii) Where available, show the degree of impact of the disease on Māori and Pacific people relative to non-Māori/non Pacific peoples. Please use age-adjusted relative risks for annual incidence rates for notifications, hospitalisations and mortality. In each instance this could include:
 - the annual number of events occurring amongst Māori, along with the age-standardised annual incidence rate per 1000 (further details below);
 - the annual number of events occurring in Pacific people, along with the age-standardised annual incidence rate per 1000;
 - the annual number of events in non Māori/non Pacific peoples, along with the age-standardised annual incidence rate per 1000;
 - both risk differences for age-standardised annual incidence rates and age-adjusted rate ratios with 95% confidence limits for Māori vs. non Māori/non Pacific peoples; and
 - both risk differences for age-standardised annual incidence rates and age-adjusted rate ratios with 95% confidence limits for Pacific peoples vs. non Māori/non Pacific peoples.
- (iii) You may include, where available, the key age-specific differences in annual incidence between Māori or Pacific people and non-Māori/non Pacific people. This may include data on particular age-sex specific groups for Māori/Pacific that have the greatest age-specific risk differences when compared with non-Māori/non Pacific people.
- (iv) Comment on the extent to which annual incidence rates (notification, hospitalisation and mortality rates) correlate with the need for pharmaceuticals.

Please use standard sources for this information (e.g. Ministry of Health mortality, hospitalisation and mental health data), supplemented where required by discussion with relevant experts and/or Medline searches. Sources could include prevalence data, incidence/notifications data, hospitalisation data, mental health episode data, and mortality data for relevant indicator(s). This could be combined with New Zealand age-ethnic-specific populations (available from <http://www.stats.govt.nz/>).

Where possible, please calculate age-standardised prevalence and annual incidence rates using the direct method against Segi's Standard World population. You can calculate age-standardised rate ratios (relative risks) and 95% confidence limits using standard binomial techniques. (Age-standardised rate ratios summarise disparity for

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Māori or Pacific people over all ages when compared with non-Māori/non Pacific, mitigating bias due to differences between ethnic groups' age structures.)

Applicants providing such information need to be aware that the use of annual incidence (notification) rates to proxy need for pharmaceuticals is valid only if survival times are similar between ethnic groups. This is often not the case with Māori and Pacific peoples, who have worse mortality and case fatality rates. In addition, hospitalisation rates do not necessarily correlate well with prevalence (here, the need for pharmaceuticals), although they might indicate the total burden of disease.

6.4.1 Background information

Historically, there have been major problems with the accuracy and validity of the data available within the New Zealand health sector, especially with ethnicity data. In general there is little relevant information available about the types of patients, disease/disability rates and pharmaceuticals used to treat primary care conditions, particularly when subdividing ethnicity by disease/indications. Mortality data are less timely than hospitalisations data and give less precise estimates of risk (because of smaller numbers of deaths). Māori death rates have in past decades been substantially under-represented because their ethnicity was often miscoded. Causes of death described on death certificates can be inaccurate. Routinely collected hospitalisations data are less accurate than mortality data-historically over one-quarter of discharge diagnoses were incorrectly coded. Because hospitals had systematically under-counted Māori admissions, there were numerator-denominator mismatches. Hospital admissions only indirectly measure need, being also affected by supply factors (such as regional variations in admitting practices relating to bed/service availability and clinical protocols). This in turn affects ethnic rates. Double counting of readmissions and of inter-hospital transfers as "new" admissions further biases the data.

Describing Māori or Pacific peoples' representation (i.e. what proportion of patients in the community are Māori or Pacific peoples) has only partial relevance to PHARMAC's decision-making. Disease prevalence does not necessarily correlate with need for pharmaceuticals. In addition, deviations from expected need for ethnic groups have little relevance if these ethnic groups are not accessing appropriate treatment. This is where accessing appropriate treatment means being able to visit a GP or similar primary care professional, the disease being identified and treatment prescribed – all equally available to non-Māori. There is evidence with many aspects of healthcare that Māori access is lower than that for non-Māori.

7. Reapplications

Key reasons for Reapplications include:

- new information has become available (e.g. clinical trials, cost-utility analyses); and
- the applicant wishes to respond to issues or questions raised by PTAC or specialist PTAC Subcommittees (as recorded in the minutes of the meeting).

7.1 Information Requirements

It is important that Reapplications address all of the concerns raised by PTAC or the PTAC Subcommittee when the original Application was considered. Please provide a list of all new information (not included in the original Application or previous reapplications) along with copies of all new information and supporting documentation.

Applicants are not required to provide copies of their original Application; however PHARMAC may contact you if this information is required.

8. Applications for the Funding of Generic or Biosimilar Pharmaceuticals

This section of the *Guidelines* should be used only for Applications to fund a generic or biosimilar pharmaceutical where the pharmaceutical, including the indication applied for, has previously been assessed by PHARMAC. If PHARMAC has not assessed other brands of the pharmaceutical, a full application is required. You can confirm this by contacting the relevant Therapeutic Group Manager at PHARMAC (++64 (04) 460-4990).

Please provide as much of the following information as possible in your Application.

8.1 Registration and Indication

Please provide a copy of the Medsafe gazette notice and approved New Zealand data sheet. If the pharmaceutical is not registered for use in New Zealand, please outline the date, or likely date, of submission of the dossier, and whether or not this would be subject to any of Medsafe's abbreviated registration processes. If known, please indicate the expected date of registration.

Please state whether there are any differences, or likely to be any differences, in the approved indications for the pharmaceutical compared with the other brand(s) of the pharmaceutical previously considered by PHARMAC.

Please also state the indication for which funding is being sought.

8.2 Pharmaceutical Information

Please provide the following pharmaceutical information:

- (i) the official or approved names of the pharmaceutical;
- (ii) available forms, strengths, pack sizes and packaging for which funding is sought (in the case of a preparation such as an aerosol, state the number of doses available from the container);
- (iii) whether there are any other available forms, strengths and pack sizes;
- (iv) if available, provide one labelled sample of the pharmaceutical inside its New Zealand packaging. If the pharmaceutical is cytotoxic or a controlled pharmaceutical, please send the sample separately directly to the PHARMAC Medical Director (note that more samples may be required later);⁵
- (v) detail any differences between the pharmaceutical and any other brand(s) of the pharmaceutical previously considered by PHARMAC, including (but not limited to):
 - differences in the efficacy, side effect and toxicity profiles;
 - differences in the pharmacokinetic profile with respect to age, concomitant pathological status, or polymorphic metabolism;

⁵ Nothing in this requirement to send samples acts as an exemption for the applicant from relevant legislation relating to the importation or distribution of pharmaceuticals, including but not limited to the Medicines Act 1981 (and its Regulations), the Misuse of Drugs Act 1975 or relevant patents legislation.

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- differences in the shelf-life;
- differences in appearance/taste (e.g., colour, shape, size, coating, ability to be split);
- difference in excipients (i.e. the inactive substances used to carry the active ingredients); and
- any other differences that the applicant is aware of that could be of relevance.

8.3 Price Information

Please provide the following price information:

- (i) the selling price (GST exclusive); and
- (ii) any alternative pricing proposals (e.g., possible price/volume trade-offs).

8.4 Market Information

Please provide the following market information:

- (i) which (if any) international markets the generic or biosimilar pharmaceutical is registered for use;
- (ii) which (if any) international markets where registration of the generic or biosimilar pharmaceutical has been declined;
- (iii) the market share of the generic or biosimilar pharmaceutical in international markets; and
- (iv) a description of uptake and acceptance of the generic or biosimilar pharmaceutical in international markets.

8.5 Patent Information

Please provide the information set out in Section 5.6 on each patent (granted and pending) that has claims relating to the generic or biosimilar pharmaceutical that is the subject of the Application, and with respect to which a claim of patent infringement could reasonably be asserted if a person, not licensed by the owner of the patent, distributed or sold the pharmaceutical in New Zealand.

If you believe that there are no relevant patents with respect to which a claim of patent infringement could reasonably be asserted if a person not licensed by the owner of the patent distributed or sold the pharmaceutical in New Zealand, please specify this in your Application.

8.6 Lead times

Please provide information about the length of time following a funding decision required to obtain sufficient stock to supply the market in New Zealand.

9. Applications for the Funding of Blood Glucose Meter Systems

This section of the *Guidelines* should be used only for Applications to fund a blood glucose diagnostic test meter and/or blood glucose diagnostics test strip where the brand has not previously been assessed by PHARMAC. We recommend you discuss your blood glucose meter system by contacting the relevant Therapeutic Group Manager at PHARMAC (++64 (04) 460-4990) before submitting your Application.

Please provide as much of the following information as possible in your Application.

9.1 International information

Please provide information advising where your brand is marketed and supplied overseas and include any relevant international regulatory evaluation reports (e.g. CE Certification or FDA approval).

Please state whether there are any differences in your brand of blood glucose diagnostic test meter and/or blood glucose diagnostic test strip that differ from the currently funded brands in the Pharmaceutical Schedule.

9.2 Product Information

Please provide the following product information:

- (i) the official or approved names of the blood glucose diagnostic test meter and/or blood glucose diagnostic test strip;
- (ii) date of notification to WAND database;
- (iii) available pack sizes and packaging for which funding is sought;
- (iv) whether there are any other available forms, strengths and pack sizes;
- (v) operating information about your brand of blood glucose diagnostic test meter and/or blood glucose diagnostic test strip, including:
 - a. coding
 - b. volume of blood required
 - c. time taken for test
 - d. temperature range of operation
 - e. open and unopened strip vial stability
 - f. strip expiry detection
- (vi) computer software information (including the ability to download information, compatibility of software and availability of software to healthcare providers and patients);
- (vii) other relevant information:
 - a. availability of an 0800 number (incl. hours of operation)
 - b. repair/replacement policy
 - c. other customer services

- (viii) if available, provide two samples of the blood glucose diagnostic test meter and/or blood glucose diagnostic test strip inside its New Zealand packaging.

9.3 Price Information

Please provide the following price information:

- (i) the selling price (GST exclusive); and
- (ii) any alternative pricing proposals (e.g., possible price/volume trade-offs).

9.4 Lead times

Please provide information about the length of time following a funding decision required to obtain sufficient stock to supply the market in New Zealand.

9.5 Product testing

Please provide local and independent testing results for your blood glucose diagnostic test meter and/or blood glucose diagnostic test strip to help determine decisions on funding of your brand by PHARMAC (and, where deemed necessary, the Pharmacology and Therapeutics Advisory Committee (PTAC) or one of the specialist PTAC Subcommittees). Details of an acceptable protocol and evaluation to compare the analytical performance of blood glucose meters on capillary whole blood samples versus reference laboratory analysis of venous plasma glucose are in Appendix 2.

PHARMAC has an agreement for the provision of services relating to capillary glucose meter studies with the Canterbury District Health Board. Details for the provider and costs are also in Appendix 2. Suppliers may opt to use this provider or opt to seek the services from an alternative local and independent provider.

Finally please supply eight samples to PHARMAC to allow separate field testing (including ease of use for all age groups) and associated computer software by the Diabetes Subcommittee of the Pharmacology and Therapeutic Advisory Committee (PTAC).

Appendix 1 – Glossary

Term	Definition
Absolute Risk Reduction (ARR) or Absolute Risk Increase (ARI)	The absolute difference in event rates between an intervention and its comparator.
Adherence	Continuation and consistency with recommended treatment regimen.
Applicant	Any person or organisation making an application to PHARMAC.
Application	An application or proposal made by a third party to PHARMAC for (a) the funding of a pharmaceutical; or (b) changes to the funding of a pharmaceutical that is already on the Pharmaceutical Schedule (e.g. a proposal to widen or restrict access).
Average cost	Total cost divided by total number of units.
Budget impact analysis (BIA)	Estimate of planned resource use and impact on budget over a period of time.
Community pharmaceutical	A pharmaceutical that is funded from the Pharmaceutical Budget and used in the community (i.e. outside of the hospital).
Combination product	Products that consist of two or more pharmaceuticals.
Combined Pharmaceutical Budget	<p>The combined pharmaceutical budget, set by the Minister of Health, includes funding for pharmaceuticals used in the community (including medical devices), cancer medicines (whether used in hospital or in the community), vaccines and some haemophilia treatments.</p> <p>The CPB does not include funding for other hospital medicines or hospital medical devices (both of which are currently funded by District Health Boards); funding for PHARMAC's operations; or payments for distribution such as the fees a community pharmacist receives.</p>
Comparator	Treatment most prescribers would replace in New Zealand clinical practice, and the treatment prescribed to the largest number of patients (if this differs from the treatment most prescribers would replace).
Confidence interval	Numerical measure of the range within which the true treatment effect is likely to lie.
Cost/QALY gained	Result of cost-utility analysis. Monetary cost per quality-adjusted life year (QALY).
Cost-benefit analysis (CBA)	Cost-benefit analysis (CBA) measures costs and benefits in monetary terms, and expresses the results as one figure representing the difference between benefits and costs ($B-C > 0$), or as a ratio (B/C).

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Term	Definition
Cost-effectiveness analysis (CEA)	Cost-effectiveness analysis (CEA) compares the relative costs of interventions against some clearly definable outcome; such an outcome may be, for example, hospitalisation days avoided, strokes prevented or hip fractures averted. The final result is a value called the incremental cost-effectiveness ratio (ICER).
Cost-minimisation analysis (CMA)	Cost-minimisation analysis (CMA) assumes that there is no net health change between different treatment options (i.e. there is no significant difference in the effectiveness of the treatments). In this case the analysis is essentially a search for the least costly alternative.
Cost-utility analysis (CUA)	Cost-utility analysis (CUA) is similar to CEA, but health outcomes are measured using a common denominator - quality-adjusted life-years (QALYs) gained. The incremental cost-utility ratio (ICUR) is defined as the change in the costs and benefits (where benefits are measured in terms of quality-adjusted life years) resulting from substituting one treatment for another.
Decision tree	Graphical representation of alternative treatments for use under conditions of uncertainty.
Diagnosis Related Group (DRG)	Patient classification scheme which provides a clinically meaningful way of relating the number and types of patients treated in a hospital to the resources required by the hospital.
Direct age standardisation	<p>Age standardisation (age adjustment) is a technique to better compare populations when their age profiles differ. It uses a weighting approach to match the age distribution of a common reference population, thereby obtaining a weighted average of age-specific rates to derive a summary event rate.</p> <p>Methods of age standardisation can be direct or indirect. Direct age standardisation gives a summary rate of events that would have been observed had the study population had the same age structure as the reference group (e.g. the number of cases of disease that would be expected if the disease rates in the study population were applied to the reference population). Further details are available in standard epidemiology texts.</p>
Direct cost	Fixed and variable costs (medical and non-medical) directly related to the treatment.
Discount rate	Rate used to convert future costs and benefits into present values (current dollars and benefits have greater value than future dollars and benefits).
Disinvestment	May involve reduction in eligibility to a treatment (i.e. tightening of access), or cessation of treatment.

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Suppliers would be responsible for providing the evaluator with sufficient consumables (including two different batches of test strips). Please note that the time frames above exclude delays outside the Christchurch Diabetes Centre's control, such as unforeseen delays in ethics approval or delays in the supply of meter testing equipment.

The supplier has the opportunity to provide the evaluator with a face-to-face instruction on the use of the meter and test strips but any additional communications with the supplier should be made via PHARMAC only to ensure the independence of the study.

Meter performance should be assessed in the ambulatory (outpatient) setting only; i.e. there is no expectation that performance should be assessed in specialist settings such as neonatal ICU.

Accuracy should be assessed relative to the reference method by Bland–Altman plots, Passing and Bablok regression analysis, and both Clarke and consensus (Parkes) error grid analyses. The results from the Christchurch Diabetes Centre will be provided to the supplier and PHARMAC.

The Christchurch Diabetes Centre will obtain regional ethics committee approval for an evaluation. The Christchurch Diabetes Centre is bound to a confidentiality agreement with PHARMAC relating to the data and information provided to it during the evaluation.

Bench-top evaluations performed to assess minor changes in performance claims for existing funded products should be discussed with PHARMAC. These will be considered on a case-by-case basis following a summary from the supplier of the proposed changes.

The paper referenced below⁶ provides full details of the methodology used by the Christchurch Diabetes Centre.

⁶ Comparison of blood glucose meters in a New Zealand diabetes centre. Florkowski et al. *Ann Clin Biochem*.2009; 46: 302-305 (<http://acb.rsmjournals.com/cgi/content/full/46/4/302>)