Guidelines

for funding applications to Pharmac

Amended 2024

PHARMAC
TE PĀTAKA WHAIORANGA

About this document

What are these Guidelines for?

These Guidelines have been written to provide information on how to submit a funding application to Pharmac (an Application) for proposed amendments to the Pharmaceutical Schedule.

Who are these Guidelines for?

These Guidelines have been specifically designed for pharmaceutical suppliers.

However, Applications can be made by anyone so these Guidelines may also be useful for other applicants in order to understand the information Pharmac requires to assess an Application.

What information is contained within these Guidelines?

These Guidelines are divided into 3 main parts as follows:

1. The process for submitting an Application
2. Further details on the information required for Applications
3. Background information and Frequently Asked Questions

When do the Guidelines apply?

Proposed amendments to the Pharmaceutical Schedule where these Guidelines apply include Applications for funding (or changing funding access to):

(i) medicines for use in the community and/or hospital;

(ii) medical devices for use in the community only - Pharmac currently funds a small number of medical devices that are used in the community; applications for Pharmaceutical Schedule listings for community medical devices need to be made in accordance with these Guidelines*;*

(iii) special foods;

(iv) vaccines;

(v) treatments for rare disorders;

(vi) generic or biosimilar medicines;

(vii) new formulations or strengths of already funded medicines;

(viii) combination products (products that consist of two or more pharmaceuticals).

Please note that the term ‘pharmaceutical’ is defined in the Pae Ora (Healthy Futures) Act 2022 as “a medicine, therapeutic medical device, or related product or related thing”. The term pharmaceutical should be interpreted for the purposes of these Guidelines to mean a medicine, including for example vaccines, generics and biosimilars or medical devices.

When don't the Guidelines apply?

These Guidelines do not apply to the following:

(i) funding applications for pharmaceuticals for individual patients through the Named Patient Pharmaceutical Assessment (NPPA) process;

(ii) Pharmac tenders, request for proposals (RFPs) or other commercial proposals issued by Pharmac;

(iii) notification of concerns regarding the safety of pharmaceuticals - Medsafe is responsible for pharmaceutical regulation and safety matters, so Medsafe should be approached in the first instance on all issues related to the safety of a pharmaceutical;

(iv) hospital medical devices - Pharmac is in relatively early stages of its hospital medical devices work and does not currently require suppliers to submit Applications as described in these Guidelines. If unsure whether the pharmaceutical needs a funding application in accordance with these Guidelines (eg. if the pharmaceutical has characteristics of a medicine and a device), please contact Pharmac for advice.

Please note that these Guidelines are subject to Pharmac's Operating Policies and Procedures and will be revised as required.

Background

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 many Applications either to fund new pharmaceuticals or widen access to pharmaceuticals that are already funded. Pharmac works within a fixed budget, so choices need to be made about which Applications should be progressed to a funding decision. This involves assessing a large amount of often complex information to identify which options would provide the best health outcomes.

Pharmac's decision-making framework sees us assessing each proposal using the Factors for Consideration (Factors), which consider health benefits, need, costs and savings, and suitability, including any impacts on other people. Using this information, all proposals are compared and ranked against one another.

In this context, 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.

The Guidelines were last fully reviewed in 2009, and further amended in 2015. These 2019 Guidelines were revised alongside the development of an online application system (PharmConnect). The main amendments include:

- Revised structure and reduced duplication of information requested – the structure of Guidelines is consistent with Pharmac's Factors for Consideration and the requirements of the PharmConnect system.
- New sections outlining additional information required in Applications for vaccines, Special Foods and community medical devices.
- Further details requested on health need; including the patient population, current treatment, and impact of disease.
- Further guidance provided on how to undertake a literature search and report the methodology and results of trials (including providing suggested table formats).
- Guidance on the ordering of clinical studies, and articles not to attach to Applications.
- Inclusion of a link to an Excel Budget Impact Assessment (BIA) template for estimating patient numbers and budget impact (along with more detailed information on how to calculate patient numbers and present a BIA).

Contents

Part 1: How to submit an Application

How to submit an Application

Applications should be submitted to Pharmac using the PharmConnect system. The PharmConnect system is an online application system that allows suppliers, clinicians and other people to submit and track the progress of funding applications.

More information and a link to the PharmConnect system can be found on the Pharmac website.

To successfully submit an Application to Pharmac , complete the following steps:

STEP 1 - register your details on the PharmConnect portal. Instructions on how to register can be found here. You must register prior to submitting an Application to Pharmac. If you are already registered, please check your contact details are correct.

STEP 2 - read and become familiar with the information required for an Application and the FAQs. All Applications must be made in accordance with the requirements of the PharmConnect system. These are also detailed within these Guidelines. Ensure you have also read Pharmac's General Terms of Listing in Section B of the Pharmaceutical Schedule and General Terms of Listing in Section H of the Pharmaceutical Schedule.

STEP 3 - contact Pharmac (+64 4 460 4990). We recommend contacting Pharmac prior to making an Application to discuss any concerns or questions you have about making an Application.

STEP 4 - complete the online application form. Include all required attachments.

STEP 5 – check all relevant information is included.

Following submission of an Application, Pharmac will undertake an initial screen of the Application to ensure 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 or information is required, Pharmac may contact the applicant and may defer consideration of the Application until any outstanding issues are resolved. If an Application, Pharmac will notify the applicant with advice on when it will be considered by Pharmac's Pharmacology and Therapeutics Advisory Committee (PTAC). PTAC Terms of Reference outline PTAC's approach to assessing Applications.

Applications for funding made outside of the PharmConnect system

Pharmac requests that all Applications are made using the online PharmConnect system. The PharmConnect system itself can be found here. In instances where it is not possible to submit an Application using PharmConnect, please contact Pharmac on +64 4 460 4990. You will be directed to the relevant member of staff who will be able to advise you on next steps.

Part 2: Information generally required in Applications

What information is needed within a funding Application?

The information that Pharmac generally requires in order to assess an Application can be grouped into different categories. These are:

1. Product Overview
2. Health Need
3. Health Benefit
4. Costs and Savings
5. Suitability
6. Economic Analysis
7. Declaration and Identification

Each of the above seven categories include several types of information needed that are identified by a series of questions. These questions are outlined in these Guidelines and in the PharmConnect application forms. A list of these questions can be found attached in this document as Appendix 1.

Note that Pharmac needs additional information specific to Applications for the funding of vaccines, special foods, and community medical devices.

We understand that different stakeholders have different resources and knowledge when it comes to making a funding application. We have therefore tailored our PharmConnect application forms to different groups of stakeholders, with different forms available for suppliers, for clinicians and for the general public.

In answering these questions, we anticipate that enough information will be provided to allow Pharmac to initiate assessment of an Application. However, it is important to note that the questions in the PharmConnect system and noted in these Guidelines are not exhaustive or prescriptive, and more or less information may be required depending on the circumstances of the Application.

Key points for preparing an Application

- Responses to questions should be thorough yet succinct. All information provided must be specific to the proposal being made.
- Use plain English wherever possible (while maintaining scientific rigour).
- Reference all sources of information used to address a question.
- Please address all the questions. If you consider a question is not relevant, or the information is not available to answer the question, please state this in your response. Justify any variations to the requested information under the relevant headings.

Associated Supporting Information

The following information should be read in conjunction with these Guidelines:

- Pharmac's Operating Policies and Procedures (OPPs)
- Factors for Consideration
- Prescription for Pharmacoeconomic Analysis (PFPA) – outlines Pharmac's preferred approach to undertaking economic analysis
- Pharmac's General Terms of Listing in Section B of the Pharmaceutical Schedule and General Terms of Listing in Section H of the Pharmaceutical Schedule

Guidance on questions

Please note:

- This section provides guidance on the questions that are asked in the supplier's application form in the PharmConnect system.
- A full list of questions that are on the supplier's application form in the PharmConnect system can be found in Appendix 1.
- The questions are listed as they appear on the supplier's PharmConnect application form.
- This guidance aims to:
    - provide clarity and to assist with understanding of the PharmConnect system's supplier application form.
    - detail the kind of information that may be relevant to provide in response the questions in the PharmConnect system's supplier application form.

Please ensure, when responding to questions, that all sources of information are appropriately referenced.

Category: Product Overview; Subcategory: Product details

Question 1: Please select the type of application you would like to make.

Question 2: If other, please specify.

Supplier applications have the following types:

- new chemical or biological entity,
- new medical device for use in the community,
- new indication for a pharmaceutical already listed in the Pharmaceutical Schedule,
- new formulation or strength for a pharmaceutical listed on the Pharmaceutical Schedule,
- treatment for a rare disorder (defined below) and
- generic or biosimilar.

If you consider your application does not fit within one of these categories, please select other and then use box 2 to describe the category that you consider best identifies the type of application you would like to make.

Question 3: If available, please detail the following codes:

If available, please detail the following codes:

- Pharmacode,
- NZMT CTPP code (New Zealand Medicines Terminology Containered Trade Product Pack),
- GTIN (Global Trade Item Number), and
- the supplier product code.

These codes are unique product identifiers that are used throughout the pharmaceutical supply chain in New Zealand. Although these codes are not required to assess an Application, they may be required by Pharmac to list an item in the Pharmaceutical Schedule.

Question 4: Have any sample(s) of the pharmaceutical been sent to Pharmac?

Question 5: If a sample has been sent, please provide information that could help us to manage the sample.

Please let us know whether you have sent a sample of the pharmaceutical to Pharmac. If a sample has been sent, information that may be useful for Pharmac staff to trace and identify the sample may include to whom the sample was addressed, the date of sending, the expected date of delivery, or any special instructions that need to be followed upon receipt and for storage of the item.

Question 6: Please attach suitable graphics, artwork or photographs in pdf or jpeg format of the following: The New Zealand packaging of the pharmaceutical, the product itself, and the product labelling.

Where available, please attach images that demonstrate the New Zealand packaging that will be used for the pharmaceutical. This information is used to help assess the pharmaceutical and informs what support may be required by patients and healthcare staff should the pharmaceutical be subsidised.

Category: Product Overview; Subcategory: Pharmacological information

Question 1: What is the registered name of the generic pharmaceutical?

Please provide the approved name of the generic chemical or biological entity as specified in the Medsafe datasheet. If the pharmaceutical is not yet registered in New Zealand, please enter the international non-proprietary name (INN). The INN is a nomenclature system used to identify active ingredients of medicines. Each INN is a unique name that is internationally consistent and globally recognised.

Question 2: What is the brand name of the pharmaceutical?

If known, please state the official brand name(s) of the pharmaceutical as specified in the Medsafe datasheet. If the pharmaceutical is not registered in New Zealand or if you are unsure of the brand name(s), please leave this question blank.

Question 3: Please provide a brief description of the principal pharmacological action of the pharmaceutical.

A brief description of the principle pharmacological action of the pharmaceutical should be provided. This should describe the biological interaction through which the pharmaceutical produces its main pharmacological effect. This may include a description of the specific molecular targets to which the drug binds, as well as the specific action that occurs there.

Question 4: Please select the appropriate category for this application.

From the list of available options, please select the category in which the pharmaceutical best falls. If you are uncertain of the appropriate category, please select ‘unknown’ from the list.

Question 5: Please provide information on the various forms, strengths, and pack sizes of the pharmaceutical that you are seeking funding for.

Please provide the following information on the pharmaceutical you wish to be funded; the forms, the strength and the pack size.

Question 6: Provide stability data for infusion treatments (if relevant).

If you are requesting funding for a treatment which is delivered by infusion, please provide the stability data that demonstrates the stability of the treatment until expiry.

Category: Product Overview; Subcategory: Proposed amendments to the Pharmaceutical Schedule

Question 1: Please provide details on the indications for which funding is sought.

Please provide details of clinical indications you would like the pharmaceutical to be funded for. In order to ensure that that we achieve best health outcomes from within the budget allocated, Pharmac sometimes targets and restricts the funded use of pharmaceuticals to specific indications or diseases. Please provide details of clinical indications you would like the pharmaceutical to be funded for. Please note that later questions request details on proposed Special Authority criteria, endorsement criteria or hospital restrictions that you would like Pharmac to consider.

Question 2: What setting will the pharmaceutical be used?

Please identify whether the pharmaceutical will be used in the community, within hospitals, or in both the community and hospitals.

Question 3: Where is the pharmaceutical likely to be used?

Question 4: If other, please specify

Please identify whether the pharmaceutical is likely to be used in operating theatres

medical wards, outpatient clinics, the community, or in other areas. If the pharmaceutical is likely to be used in other areas, please list these areas.

Question 5: Please provide a summary statement of the main therapeutic claims of the pharmaceutical and its proposed use.

Please provide details of the therapeutic claims relating to the pharmaceutical. Information on the therapeutic claims in relation to the pharmaceutical will help determine the clinical advice that would be sought in relation to the pharmaceutical and help determine where in the Pharmaceutical Schedule any listing could occur.

Category: Product Overview; Subcategory: Dose

Question 1: Please provide details on the course of treatment that would be likely used in New Zealand clinical practice for each indication for which funding is requested. This should include both the dose and the duration of treatment.

Please provide information on the dose for each indication that the medicine is likely to be used to treat. The information provided should be based on the most likely dose regimen used in New Zealand clinical practice. In the case of a pharmaceutical that is not used for chronic therapy, please also provide information on the average length of a treatment course and anticipated frequency of repeat courses of treatment.

A suggested table format of the estimated number of packs dispensed based on most likely dose regimen in New Zealand clinical practice can be found below:

Table X: Estimated packs dispensed

Dose*	
Dosing frequency	
Average length of a course of treatment	
Anticipated number of repeat courses of treatments	
Estimated number of packs dispensed**	

* based on most likely dose regimen used in New Zealand clinical practice

** in the case of multiple pack types, add another line for each type of pack

Question 2: Is the dosage recommended in the New Zealand data sheet, or the dosage regimen used in the pivotal trials, different from the dosage regimen likely to be used in NZ clinical practice? If so, please provide details.

Is the dosage regimen that would likely be used in New Zealand clinical practice different to the recommended dosage regimen (as indicated in the Medsafe datasheet) or different to the dosage regimes used in the pivotal trials? If so, please provide details of the differences between the dosage regimens and explain the rationale for this difference.

Question 3: Do you have any post-marketing data on dosage in clinical practice? If so, please provide details.

In addition, if available, please include any post-marketing data on dose used in clinical practice. Post-marketing data on dosages used in clinical practice can help us to accurately predict the impacts of funding the pharmaceutical.

Category: Product Overview; Subcategory: Regulatory status of the product

Question 1: Has the pharmaceutical been registered with Medsafe for all indications for which funding is sought?

We consider funding applications relating to medicines that have been either Medsafe registered or submitted to Medsafe for funding. Funding applications for unregistered medicines are typically only considered on a case-by-case basis.

If the pharmaceutical is not registered for all indications, please contact Pharmac to discuss before submitting your application.

Unregistered medicines

For the medicine or indication to be considered by Pharmac for funding in parallel with Medsafe assessment, there needs to be an active application lodged with Medsafe and evidence of payment of Medsafe application fees should be provided.

Medicines for rare disorders

Suppliers of medicines for rare disorders do not need to have submitted applications to Medsafe for regulatory approval before applying to Pharmac, but the pharmaceutical must be Medsafe-approved before a final decision can be made to fund it.

Funding applications for medicines for rare disorders, where Medsafe approval or submission has not yet occurred, need to meet the following three policy principles:

1. Medsafe, or an approved international regulatory authority, has approved the medicine for the specific indication or condition.
2. The disorder is a clinically defined disorder that affects an identifiable and measurable patient population of less than 1:50,000 in New Zealand.
3. The medicine is only registered to treat the rare disorder. If it is registered for other disorders (or is part of phase three clinical trials for other disorders), those other disorders must meet principle 2.

Question 2: Please provide details of the Medsafe registered indications.

Please provide details of the registered indication(s) for the pharmaceutical, as specified in the Medsafe datasheet. Please include the link to the Medsafe datasheet. If a Medsafe datasheet is not available (e.g. for medicines to be considered in parallel with Medsafe approval or for rare disorders), please provide details of the indication for which Medsafe registration and funding is sought.

Question 3: Please attach the Medsafe approved datasheets (if available).

Medsafe is the New Zealand Medicines and Medical Devices Safety Authority. It is a business unit of the Ministry of Health and is the authority responsible for the regulation of therapeutic products in New Zealand. Further information on Medsafe can be found at www.medsafe.govt.nz.

Question 4: Are other formulations of the pharmaceutical registered for use in New Zealand? If so, please provide details.

Please provide details of any other registered formulations of the pharmaceutical in New Zealand. In your response, please include details of the formulation, the brand name, and the strength.

Question 5: Please provide information on other OECD (Organisation for Economic Co-operation and Development) countries where registration has been sought and approved. In your response, please indicate whether there are any variations in registered indications between New Zealand and the other country and if any boxed warnings are associated with the pharmaceutical.

Please provide the following details as applicable; jurisdiction, variations in registered indication, and details on warning.

Question 6: Please provide information on other OECD countries where registration has been sought and declined. Please provide information on the reasons for decline.

Please provide the following details as applicable; jurisdiction, variations in registered indication, details on warning, and reasons for decline.

Question 7: Provide details of other presentations or formulations of the pharmaceutical that have been submitted for approval, or are already approved, in other OECD countries.

Please include details on the formulation, strength, and where applicable, differences in indication.

Question 8: Are you aware of the pharmaceutical being used to treat any other indications other than the registered indications? If so, please provide details.

Please detail indications other than the New Zealand registered indications that the pharmaceutical may be used for. For example, if the pharmaceutical is registered overseas for alternative indications, or if the pharmaceutical has been used for alternative indications in clinical practice.

Question 9: For unregistered products or indications, please provide details of when the applications for registration was filed with Medsafe. If known, please indicate the expected date of registration.

Pharmac requires that pharmaceuticals are registered with Medsafe before a final funding decision is made. For unregistered products or indications, please provide details on when the applications for registration was filed with Medsafe. If known, please indicate the expected date of registration.

Question 10: If the Medsafe registration document is unavailable, please attach copies of relevant Food and Drug Administration (FDA) and/or European Medicines Agency (EMA) assessment.

If the Medsafe registration document is unavailable, please attach copies of relevant Food and Drug Administration (FDA) and/or European Medicines Agency (EMA) assessment (note that these reports only need to be attached for unregistered pharmaceuticals).

Category: Product Overview; Subcategory: Patent information

Question 1: Please list all relevant granted and pending patents (in order of relevance) that have claims relating to the pharmaceutical that is the subject of the Application, including the patent number and patent expiry date. These may include chemical, formulation and relevant indication patents.

Please provide information on patents that may impact on the use and supply of the pharmaceutical within New Zealand. Information that we require includes;

- the patent number.
- the patient expiry date.
- the type of patent (e.g. drug substance, finished drug product, method of use, ‘Swiss-type’ claim).
- the patent owner.
- if you are not the patent owner, details of your right to sell or distribute the pharmaceutical in New Zealand.
- 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 in New Zealand and who is authorised to receive notices related to the patent.

These patents may include, but are not limited to, patents relating to active pharmaceutical ingredients, indications, formulation etc.

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 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.

Category: Health Need; Subcategory: The disease and its impacts

Question 1: Please provide an overview of the disease that would be treated by the proposed pharmaceutical.

Please provide an overview of the disease for which funded treatment is sought.

Details that you may wish to provide in this overview might include risk factors for developing that disease, diagnosis, symptoms, and prognosis of disease.

If you are requesting that the use of the proposed pharmaceutical is restricted to specific subgroup(s) of the New Zealand population with a disease, please indicate whether the usual course of the disease differs for that subpopulation when compared to others with the disease.

Please do not describe the impact that the proposed pharmaceutical has, but rather limit to information on the disease or condition and its impact on the patient.

Please reference all the sources of information that you used to inform your overview.

Question 2: How unwell is a person with the disease? Please provide details on the severity of symptoms experienced by the average patient.

One of Pharmac's Factors for Consideration is the health need of the person. One way in which we consider this is by comparing life expectancy and quality of life at full health, to life expectancy and quality of life for the average patient with the disease who is likely to be treated with the pharmaceutical. It therefore looks at the loss of health that the disease causes despite the currently funded treatments in New Zealand. This includes both the loss of length of life despite current treatment, as well as any reduction in health-related quality of life despite current treatment.

It's important to be aware that health need is independent of the efficacy of the pharmaceutical that is the subject of the Application. The responses in this section should be limited to discussing the health needs of the average patient with the disease despite currently available treatments.

When discussing the impact of a disease on the patient, please consider the following questions:

- What areas of health-related quality of life may be impacted? Areas of health-related quality of life include (but are not limited to) pain and discomfort, disability (e.g. physical functioning), and psychosocial issues (e.g. anxiety, depression, social functioning).
- To what extent, or how severely are these areas impacted?
- Is the disease associated with premature mortality?

Please reference all sources of information.

For further information on the tools that Pharmac uses to assess quality of life, please see the Prescription for Pharmacoeconomic Analysis.

Question 3: Does this disease or condition have an impact on patient health-related quality of life? If so, please provide details on areas of health-related quality of life that are likely to be impacted and severity.

Question 4: If possible, please provide information on the total undiscounted quality-adjusted life year (QALY) loss associated with the disease. In other words, what is the QALY of patients with the disease compared with the QALY of the same age specific population in perfect health).

The quality-adjusted life years (QALY) is a summary measure of health outcomes.

It depicts both the quality and quantity of life lived. One QALY is equal to living one year in full health.

Please provide information on the loss of health associated with the disease (i.e. the QALY loss without treatment). This is the QALY of patients with the disease compared with the QALY of the same age-specific population in perfect health.

For details on how to calculate QALYs, please refer to Pharmac's Prescription for Pharmacoeconomic Analysis.

Question 5: Does the disease impact on the health of the patient's family, whānau or wider society? Please explain and provide sources of information.

Some diseases may affect people other than the person who has the disease. For example, during pregnancy, a disease may affect the unborn child as well as the mother, or a communicable disease may have an impact on wider society.

One of Pharmac's Factors for Consideration is the health need of others and we think about the impact of the disease on people beyond the patient.

Please describe the impact of the disease on family, whānau, and wider society.

All significant health effects are relevant. Examples of the impact of disease on others might include:

- the risk of infection for those caring for patients with an infectious disease
- carer burden associated with conditions with high dependency on others (for example, blindness, Prader-Willi syndrome, dementia, intellectual disabilities, neurodevelopmental disorders)
- the impact of smoking on children exposed to second-hand smoke
- the impact of alcohol and drug exposure in pregnancy on unborn babies
- the impacts on partners, families and others (including children) of challenging behaviours caused by psychosis and/or exacerbated by alcohol and drug use
- the impact of sexually transmitted infections on sexual partners.

While quantitative data is preferable, qualitative research is also useful.

Please provide details on all sources of information.

As with all questions in the Health Need section, please limit your response to the impact of the disease or condition and do not discuss the pharmaceutical that is the subject of the Application.

Question 6: What is the impact of the disease on Māori health outcomes?

Pharmac considers how decisions may impact on the health outcomes of Māori in accordance with its te Tiriti o Waitangi (Treaty of Waitangi) obligations.

Please describe whether:

- the condition disproportionately affects Māori
- there are any differences in progression of disease for Māori
- there are delayed treatment issues
- there are inequities in access to treatment
- or whether the condition affects Māori significantly more than it does other New Zealanders.

Where feasible, please quantify the disease or disability incidence, prevalence and mortality rates for Māori compared with other New Zealanders.

Sources of data may include:

- Te Whaioranga
- Tatau Kahukura: Māori Health Chart Book
- Unequal Impact II (studies on cancer statistics)
- Māori Health Review
- NZ Burden of Disease, Injuries and Risk Factors Study 2006-2016
- Annual Update of Key Results: New Zealand Health Survey

Please reference all sources of information used in your response.

Please limit your response to the health need of Māori in relation to the disease in question, do not discuss the pharmaceutical that is the subject of the Application.

Question 7: Does the disease fall into one of the categories of Pharmac's Māori health areas of focus? Please explain and provide sources of information.

Please identify whether the disease for which funding has been sought has been identified as one of Pharmac's Māori health areas of focus. The Māori health areas of focus are detailed in Te Whaioranga - Pharmac's Māori Responsiveness Strategy.

Question 8: Does the disease disproportionately affect population groups that may already be experiencing a health disparity?

As part of its Factors for Consideration, Pharmac considers the impact that a pharmaceutical could make for population groups that are already experiencing a health disparity.

Please provide details on whether the disease disproportionately affects population groups that may already be experiencing a health disparity. Please include a description of the impact of the disease on the identified population.

Pharmac defines health disparities as 'avoidable, unnecessary and unjust differences in the health of groups of people'. Population groups experiencing health disparities will have one or more shared characteristics that mean they experience poorer health outcomes as a result of broader systemic social determinants of health. This disadvantage may mean that the

population group may be more susceptible to a given illness, or may experience poorer health outcomes, than the average person with the illness.

Population groups that have previously been identified that may experience health disparities in New Zealand include Pacific peoples, refugees, people living in areas of New Zealand that have been identified to have New Zealand deprivation index scores of levels 9-10; and sub-regionally deprived populations (geographical areas in New Zealand where residents face significantly greater health disparities than other geographical areas).

Sources of data may include:

- Tupu Ola Moui: Pacific Health Chart Book 2012
- Annual Update of Key Results: New Zealand Health Survey

Please reference all sources of information used in your response

Question 9: Is the disease a Government health priority? If yes, please indicate which category the disease falls into.

We need to make sure our funding decisions help achieve the overall health priorities identified by the Government. Where relevant, please provide information where the disease, condition or illness is a government priority.

In line with Pharmac's Operating Policies and Procedures, the Government health priorities have been selected from strategic health sector documents.

Further information and the current list of Government health priorities can be found here.

Category: Health Need; Subcategory: Patient population

Question 1: Who is the target population?

Please describe the New Zealand population that would be treated with the pharmaceutical. Details you may wish to provide include age of disease onset, age of diagnosis, ethnicity, prevalence or incidence in other populations experiencing disparities, important comorbidities, and life-expectancy.

Please reference all the sources of information you used to answer this question.

Question 2: What is the prevalence and incidence of the disease in New Zealand? Please provide estimates of the number of people in New Zealand who have the disease, the number of Māori in New Zealand with the disease and the number of Pacific people in New Zealand with the disease, and how these numbers change over time (this data can be provided in the epidemiology table template).

Please also provide details on how these numbers were obtained and how they are applicable to the New Zealand setting.

For each requested indication(s), please provide estimates of the:

(i) number of people (in New Zealand with the particular condition(s)

(ii) number of Māori people in New Zealand with the particular condition(s)

(iii) number of Pacific people in New Zealand with the particular condition(s)

(iv) morbidity associated with the condition (e.g. annual number of hospitalisations)

(v) premature mortality associated with condition in New Zealand (e.g. annual number of deaths, stratified by age group; number of potential years of life lost compared with a health person of the same age).

All prevalence estimates and assumptions should be obtained systematically. Please supply details of the search strategies used to identify these.

If New Zealand specific data is available, this must be provided. If New Zealand specific data is not available, discuss whether the data provided is representative of the New Zealand setting. If data needs to be extrapolated, please clearly and explicitly outline assumptions and sources. Include percentages and means with estimates of uncertainty (eg interquartile range, standard deviation and ranges) for these data, where possible.

Sources of incidence and prevalence data include:

- Medline
- Medscape
- UpToDate
- Ministry of Health (Mortality Records, New Zealand Health Survey, Hospital Event data)
- Global Burden of Disease (GBD) study
- World Health Organization (WHO)

Question 3: Please attach the completed Epidemiology Data template.

Please attach a completed epidemiology template. The epidemiology template can be found here.

Category: Health Need; Subcategory: Current treatment options

Question 1: What treatments are currently used in New Zealand to treat the disease? Please describe the current treatment algorithm for the target population and if possible, include a flowchart illustrating the current management of the disease in the target New Zealand population.

Please provide details of the comparator treatments available in New Zealand. These are the likely treatments (including non-pharmaceutical treatments, for example surgery, radiation and physiotherapy) a person with the disease would currently receive in New Zealand.

If the pharmaceutical can be used for several diseases and there are different treatments, please provide the likely treatment algorithm for each disease. If there is currently no treatment available, or if palliative care would be the recommended approach, please indicate this in your response. Consideration should be given to whether current treatment differs for different target population. In such cases information should be provided on treatments most likely to be replaced in the targeted population(s).

If possible, include a flowchart illustrating the current management of the disease in the target New Zealand population.

This should be the funded treatment(s) most likely to be replaced in New Zealand clinical practice and/or the treatment given to the largest number of patients (if this differs from the treatment most prescribers would replace).

Question 2: What sources of evidence were used to inform the current treatment algorithms that you have provided?

Please provide details of the sources you used to inform your response to question 1 above.

Question 3: Please provide commentary around how well the current treatments work for the disease being treated. In your response, please also provide details of any risks or any tolerability issues associated with the current treatment options.

Question 4: Are there any issues regarding the availability or suitability of current treatments for this indication?

Please detail any existing non-clinical issues there are with current treatment options. These issues may include issues with availability, taste, size, the practicality and appropriateness for the target population (for example, the suitability of current treatment in groups such as children).

Please provide details on the availability of current treatment. This may include details on whether the current treatment is listed on the Pharmaceutical Schedule for the relevant indication or used as part of standard practice within DHB hospitals.

For further information on availability and suitability, please click here.

Question 5: What is the recommended dose of current treatments and dose equivalencies between current treatment and the proposed pharmaceutical?

Question 6: What is the shelf life of the current treatment? Please provide an indication of how this compares to the shelf life of the proposed pharmaceutical.

Please provide details of the shelf life of the current treatment options and provide information on how this compares with the shelf life of the proposed pharmaceutical.

Question 7: How would the proposed treatment change the current treatment algorithm? Please include a flowchart illustrating the expected changes in clinical management.

Please provide a diagram that demonstrates how the proposed pharmaceutical would impact the treatment of the relevant indications and describing other pharmaceuticals, if any, likely to be prescribed for use with the pharmaceutical as part of a course of treatment.

Summarise the differences between the current and proposed clinical management, as depicted in the algorithm(s).

Ensure that the population and main comparator(s) in the clinical management algorithm and relevant to the New Zealand population and are consistent with those previously described.

Please provide details on the sources of evidence used to inform the current treatment algorithm. This may include:

(i) a literature review of relevant published clinical management guidelines (preferred method). Pharmac prefers independent, up-to-date evidence-based clinical practice guidelines developed for New Zealand or relevant to the New Zealand setting. Include a copy of the literature review and guidelines as an attachment to the application.

(ii) an expert panel and/or a well-designed survey (if current clinical management guidelines are not available). Present details of who the survey was sent to, who responded, and the survey questions and responses in an attachment to the submission.

Category: Health Benefit; Subcategory: Identification and selection of studies

Question 1: How was the literature searched? Please provide details on the search strategy that was used to retrieve clinical studies.

Please provide information on how the literature you have provided with your application was sourced by providing details on your search strategy. Please detail the inclusion criteria and limits that were placed on the search.

All evidence should be obtained systematically. The primary objective of the literature search is to locate all published randomised controlled trials (RCTs) that directly compare the intervention with one or more relevant comparators within the relevant indication(s). Meta-analyses and systematic reviews are also of relevance.

If direct randomised trials comparing the proposed medicine with the main comparator are not identified, search again separately for randomised trials of either the proposed medicine or the main comparator. Present both search strategies. If neither of these searches retrieve suitable results, broaden the original search to identify all nonrandomised studies of the proposed medicine. This includes cohort studies, case-control studies and quasi-experimental studies.

Details of the search strategy used to retrieve clinical studies should be described, including the:

(i) databases and trial registries searched (e.g. PubMed);

(ii) date the searches were done;

(iii) time period searched;

(iv) any limits placed on the searches;

(v) research question;

(vi) search terms, including keywords or MeSH headings used.

It is important that search strategies define the research question and use the correct search terms. Pharmac recommends the use of the PICO framework (Population, Intervention, Comparison, Outcome) to help with the correctly frame the research question and identify search terms.

Please also provide details of any supplementary or opportunistic searches undertaken.

Please note that search strategies do not need to be restricted to the English language, however you must provide a reputable translation for articles that are not in English.

Question 2: What inclusion and exclusion criteria were used in the selection of studies?

Please describe the inclusion and exclusion selection criteria that were used in your search strategy.

A suggested table format is provided below.

Table [X] Eligibility criteria used in the search strategy

	Inclusion criteria	**Exclusion criteria**
Population		
Intervention(s)		
Comparator(s)		
Outcomes		
Settings (if applicable)		
Study design		
Other search limits or restrictions applied		

Question 3: Please provide a flow diagram of the number of studies included and excluded at each stage.

A flow diagram of the numbers of studies included and excluded at each stage should be provided, such as the Preferred Reporting Items for Systematic Reviews and Meta-Analyses (PRISMA) flow diagram. Further information on PRISMA can be found at the PRISMA website here.

Question 4: What studies met the inclusion criteria?

Please provide a list of all studies that meet the inclusion criteria. The studies should be identified as either:

1. Randomised controlled trials (RCTs)
2. Meta-analyses and systematic reviews
3. High quality cohort and case-control studies

All studies that meet the inclusion criteria should be attached to the application.

If you are submitting an application outside of the PharmConnect system, please provide a list of all studies that meet the inclusion criteria. The studies should be ordered as follows:

1. Randomised controlled trials (RCTs)
2. Meta-analyses and systematic reviews
3. High quality cohort and case-control studies

Within each of three categories above, please order the studies by date of publication (with the most recent first). A suggested table format is provided below:

Table X: List of all relevant studies

Study reference	**Full reference of report**
Randomised controlled trials	
Meta-analyses and systematic reviews	
High quality cohort and case-control studies	

Please also include all supplements, errata, editorials, study protocol publications, and journal correspondence relating to the pivotal published trials (these should be attached after the trials). Do not attach studies that do not meet the inclusion criteria. Clinical study reports do not need to be provided, however please have these available upon request for the pivotal RCTs.

Note that Pharmac has considered and will continue to consider all levels of evidence. However, we will be most influenced by the results of direct randomised trials as the most rigorous source of data.

Trials Used in Meta-Analyses
For any relevant trial identified from a meta-analysis, include the individual trial report.

Clinical Study Reports
Clinical study reports do not need to be provided, however please have these available upon request for the pivotal RCTs.

Unpublished and Ongoing Trials
Please provide details of all unpublished and ongoing studies that should provide additional evidence in the next 12 months for the indication being appraised. This can be in the form of a print-out from www.clinicaltrials.gov.

If including unpublished articles, specify why each trial has not been published and their expected dates of publication (if applicable).

Abstracts and posters are not usually appropriate sources for descriptions of the study methodology or primary outcomes of studies. However, if they are adequately detailed, they may be used as references to update information after the primary analyses or any analyses of secondary outcomes not detailed in the published report.

Articles Not to provide in Applications
Please do not attach copies of the following:

- phase 1 clinical trials;
- studies that do not meet the inclusion criteria;
- narrative reviews;
- reviews of the disease;
- studies outlining the biological rationale for the treatment;
- information unrelated to the pharmaceutical and disease under consideration;
- expert opinion.

Question 5: What studies were identified in the literature search and which were excluded? Please provide a citation list of all identified studies, and indicate which trials were excluded and the basis for their exclusion.

All appropriate evidence relating to the pharmaceutical and target population should be identified and presented. Please provide a citation list of all identified studies, and indicate which trials were excluded and the basis for their exclusion.

Question 6: Please attach all identified randomised controlled trials that meet the inclusion criteria.

The studies should be ordered by date of publication (with the most recent first).

Question 7: Please attach all identified meta-analyses and systematic reviews that meet the inclusion criteria.

For any relevant trial identified from a meta-analysis, include the individual trial report.

Question 8: Please attach all identified high quality cohort studies and case-control studies that meet the inclusion criteria.

Question 9: Please attach all supplements, errata, editorials, study protocol publications, and journal correspondence relating to the pivotal published trials.

Question 10: Please attach a register of all ongoing trials that should provide additional evidence in the next 12 months.

This can be in the form of a print-out from clinicaltrials.gov. If there are no ongoing studies, please state.

Category: Health Benefit; Subcategory: Trial design and characteristics

Question 1: Please provide a summary of the methodology for each of the pivotal clinical trials.

Please provide a summary of the methodology of the pivotal trials. At a minimum, the information requested in PICO (Participants, Intervention, Comparison, Outcome) should be included.

A suggested table format for the reporting of study methodology is presented below:

Table [X] Summary of trial methodology.

	Study Reference
Trial design (including details of blinding and randomisation, if applicable)	
Eligibility criteria used to select trial participants (e.g. medical condition, age, sex, etc.).	
Settings and locations where the data were collected	
Intervention(s) including dose, method of administration, dose timing and frequency, and titration schedule where appropriate (N enrolled)	
Comparator(s) including dose, method of administration, dose timing and frequency, and titration schedule where appropriate (N enrolled)	
Permitted and disallowed concomitant medication	
Primary outcome measures (including units of measurement and timing of assessments)	
Secondary outcomes measures (including units of measurement and timing of assessments)	
Pre-planned subgroups	

Question 2: Please describe the characteristics of the participants for each of the pivotal trials.

In a table, please describe the characteristics of the participants at baselines for each of the pivotal trials. Provide details of baseline demographics, including median age, sex, and relevant

variables describing disease severity and duration, and appropriate previous treatments and concomitant treatment. Highlight any differences between trial groups.

A suggested table format is presented below:

Table [X] Characteristics of participants in the studies across treatment groups

Baseline characteristic	Treatment group X	Treatment group Y	[Add more columns as needed]
Trial 1 (n=[x])	(n=[x])	(n=[x])	(n=[x])
Age			
Sex			
[Add more rows as needed]			
Trial 2 (n=[x])	(n=[x])	(n=[x])	(n=[x])
Age			
Sex			
[Add more rows as needed]			

Adapted from NICE Single technology appraisal: User guide for company evidence submission template, April 2017
https://www.nice.org.uk/process/pmg24/chapter/instructions-for-companies

If a CONSORT diagram is not included in the published study, please include a diagram in the application showing the flow of participants through each stage of each of the pivotal trials. Include patients evaluated for enrolment, those assigned to a treatment category, patients who received treatment as allocated, patients who completed follow-up, and patients included in the main analyses. Provide details of and the rationale for participants who crossed over treatment groups, were lost to follow-up, or withdrew from the RCT. Further information on CONSORT diagrams can be found here.

Category: Health Benefit; Subcategory: Trial results

Question 1: What were the outcomes and methods of analysis in the pivotal trials? For each pivotal trial, please create one record. Please add additional entries as required.

This is your opportunity to provide the evidence which supports your application. Please provide a concise accurate summary of each of the pivotal trials.

For each of the pivotal trials, describe the relevant outcomes, including the definition of the outcome, and method of analysis. Outcomes may include mortality, morbidity, function and health-related quality of life. A suggested table format is included below.

Table X: Methods of data collection and analysis of *[outcome]*

Study reference	Outcome definition	Method of analysis

Question 2: What did the pivotal trials demonstrate? Please provide a summary of the study results for each relevant comparison and outcome.

Please provide a summary of the study results for each relevant comparison and outcome. The summary may include information that is presented graphically to supplement text and tabulated data, but graphs should not be used as an alternative to text and data. A suggested table format is presented below.

Table X: Results summary for [outcome]

Study reference	Outcome intervention n/N (%)	Outcome Comparator n/N (%)	Absolute difference (95% confidence interval) (p value)	Relative difference (95% confidence interval) (p value)

Adapted from EUnetHTA Evidence submissions templates (here) to support core HTA information and rapid assessments: Pharmaceuticals evidence submission template long version, October 2015

Please order studies by comparison, e.g. all studies comparing the intervention with comparator x are listed first, followed by all studies comparing the intervention with comparator y. A study may appear in the table more than once if it has more than two treatment arms

Data should be presented according to intention-to-treat whenever possible. Alternative presentations of the data should be justified.

For dichotomous outcomes, the results ideally should be expressed both as relative risks (or odds ratios) and risk (or rate) differences. Both absolute and relative data should be presented. If outcomes were measured at more than one time point, justify why that endpoint was selected and discuss whether the treatment effect differs across other time points. The method for analysing time-to-event analysis should be described. Where the analysis is based on a Cox proportional hazards model, present the hazard ratios and their 95% CIs.

In the case of survival analyses, all Kaplan-Meier curves (i.e. product limit) should provide the number of patients at risk at various time points.

When interim data are quoted, this should be clearly stated, along with the point at which data were taken and the time remaining until completion of the trial. Analytical adjustments should be described to cater for the interim nature of the data.

Discuss and justify any clinically important differences in the results between the different arms of a trial and between trials.

Question 3: Are there any factors that may influence the applicability of clinical study results to patients in routine clinical practice in New Zealand? Would the same clinical benefits and adverse effects be expected?

When assessing an application, Pharmac needs to understand whether there is anything that may affect whether the results seen in the evidence will occur in clinical practice. For example, if the proposed pharmaceutical is taken with an adjuvant therapy that is not available in New Zealand, this should be highlighted and the potential effect of this should be discussed.

Describe any factors that may influence the applicability of clinical study results to patients in routine clinical practice in New Zealand. Consider the geographical and clinical setting of the

studies and how these and other factors could affect the reproducibility of outcomes when the pharmaceutical is used in routine clinical practice. Factors that may differ between the trial and what would be expected were the proposed pharmaceutical funded according to the requested restriction include:

- Patient populations (e.g. age, ethnicity, performance status, previous treatments);
- Disease (e.g. disease severity);
- Clinical management (e.g. dose schedule of comparator, permitted/disallowed concomitant drugs, monitoring or assessment frequency).

An example table is provided below.

Table X: Example differences in factors between the trial setting and the New Zealand setting

Characteristic	**Trial setting**	**New Zealand setting**	**Conclusion**	**Reference**
Disease or condition severity	42% stage I or II, 58% stage III or IV	65% stage I or II, 35% stage III or IV	[discuss whether this factor is likely to result in a difference in treatment effect, safety or patient management]	[reference for source of evidence]
Concomitant treatment	Cisplatin 75 mg/m2 every 4 weeks for 6 cycles	Carboplatin 360 mg/m2 every 4 weeks for 6 cycles		
Health care system	United States and Japan	New Zealand		
Age	Average age 54, 4% older than 80	Average age 61, 12% older than 80		

Adapted from Guidelines for preparing a submission to the Pharmaceutical Benefits Advisory Committee, Version 5.0, September 2016, here.

Describe the relevance of the outcomes assessed in clinical studies to clinical benefits and adverse effects expected in New Zealand health sector and pharmaceutical funding environment. Please reference all sources of information.

Question 4: Please identify, discuss and justify any clinically important differences in the results between the different arms of a trial and between trials.

Question 5: Does the pharmaceutical have similar, greater, or fewer side effects and/or toxicity when compared to current treatment options? Please provide details.

The preferred data source for evidence on clinical adverse effects are comparative randomised controlled trials. However, findings from non-comparative trials may sometimes be relevant and should be included (for example, post-marketing surveillance data or placebo-controlled trials). If a further literature search is undertaken, please provide details of the search strategy, and methodology and results of the studies (as outlined in section X). Please attach all relevant sources of evidence.

In a table, tabulate the total number of adverse events, frequency of occurrence (as a %), absolute and relative risk and 95% CI reported in each of the clinical studies. Categorise the adverse events by frequency, severity and system organ class. A suggested table format follows.

Table X: Overview of adverse events

	Study reference				Study reference			
	Intervention (n = x) n (%)	Comparator (n = x) n (%)	Rel risk (95% CI)	Risk diff (95% CI)	Intervention (n = x) n (%)	Comparator (n = x) n (%)	Rel risk (95% CI)	Risk diff (95% CI)
Total number of adverse events								
Total number of serious adverse events								
Total number of deaths								
Total number of adverse events leading to temporary or permanent treatment withdrawal								
Total number of withdrawals from the study because of adverse events								
Adapted from EUnetHTA Evidence submissions templates (here) to support core HTA information and rapid assessments: Pharmaceuticals evidence submission template long version, October 2015								

Discuss the results of the studies, and the type and frequency of adverse events that might be expected in clinical practice with the pharmaceutical in the indication.

Pharmac is specifically interested in comparative safety. Please provide a brief summary of any additional safety issues for the pharmaceutical compared to the relevant comparator if used in New Zealand clinical practice for the indication.

Question 6: What impact does the proposed pharmaceutical have on patient-reported outcome measures?

Patient-reported outcome measures include generic or condition-specific measures of quality of life, symptoms or function. These may also include multi-attribute utility instruments (MAUIs), in which the scoring method for the instrument is anchored on a quality-adjusted life year scale of 0 (death) to 1 (full health).

Several commonly used MAUIs are the Health Utilities Index (HUI2 or HUI3), the EQ5D-3L or -5L ('EuroQol'), the Short Form 6D (SF-6D). Pharmac currently uses the EQ5D-3L for the mapping of utility values, however if alternative MAUI are used in clinical studies, these will be considered. For further information on how Pharmac measures health-related quality of life and maps utility values for use in cost-utility analyses, please refer to the Estimating Health Benefits section of the PFPA which can be found here).

If a patient-reported outcome measure is used within the study, please describe:

(i) the measure used;

(ii) the timing of assessments, including how often and at what points in the study the instruments were administered;

(iii) who administered the questionnaire and in what setting;

(iv) the power of the study – whether it was powered to detect a clinically meaningful change in quality of life as a continuous measure (as distinct from being powered to simply detect a change in dichotomous of continuous clinical or surrogate endpoints).

(v) the characteristics of the patients who missed or refused to complete patient-reported outcome measures and compare them with those patients who completed the

questionnaires. Report the number of patients eligible for the questionnaire and the number of patients who responded for each time point.

Report results (with 95% CI) for each time point and each arm within the trial.

If more than one measure has been used in the included study, compare the results from all of them.

Please attach report(s) detailing the methodology and results for assessing the impact of treatment on patient-reported outcomes measures within a trial (if this differs from the published trial report).

If the measure used in the clinical study differs from the EQ5D-3L, please provide a mapping to the EQ-5D (if possible), with details on the methodology used for mapping.

Discuss the interpretation of these results. Assess the results against other outcomes measured in the trial.

Category: Health Benefit; Subcategory: Interpretation of the evidence

Question 1: Please provide a general interpretation of the evidence base, considering the clinically significant health benefits and potential health losses to the patient that could be gained from the pharmaceutical, relative to those of the comparator.

Pharmac makes decisions based on the available evidence. When considering whether to fund a proposed pharmaceutical, we need evidence to demonstrate how funding it would allow us to get the best health outcomes.

Please provide a general interpretation of the evidence base, considering the clinically significant health benefits to the patient of the pharmaceutical, relative to those of the comparator(s). This may include both improvements in extension of life and health-related quality of life.

As well as health benefits, also consider any potential health losses because of the funding decision. This includes harm done by adverse effects as well as no longer providing a gain that current treatment delivered. Please reference all sources of information

For further information on how Pharmac measures and considers health benefits to the patient, please refer to the Factors For Consideration.

Question 2: If available, please provide details of the incremental health benefits associated with the proposed pharmaceutical when compared to the comparator treatment. Where available, this information should be presented in the form of quality-adjusted life year (QALY) gains.

When considering a pharmaceutical, we need to understand how much benefit it could provide an individual. Pharmac uses quality-adjusted life years, or QALYs, to express this benefit. Along with a narrative on the health benefits of a pharmaceutical to the patient, information can be provided on the quality-adjusted life year (QALY) gains of the proposal.

For further information on how Pharmac measures health-related quality of life and maps utility values for use in cost-utility analyses, please refer to the Estimating Health Benefits section of the PFPA which can be found here.

Question 3: Please provide a general interpretation of the evidence base, considering the clinically significant health benefits and potential health losses that could be gained from the

pharmaceutical, to the family and whānau of the person receiving the treatment, and to wider society.

Some diseases and conditions may affect people other than the person who has the condition. For example, during pregnancy, a disease may affect the unborn child as well as the mother or a communicable disease may have an impact on wider society.

Please provide a general interpretation of the evidence base, considering the clinically significant health benefits (and losses) to the family, whānau, and wider society of the pharmaceutical, relative to those of the comparator(s). This includes the health benefits to carers. Please reference all sources of information.

Examples of treatments associated with health benefits to others include:

- treatments for infectious diseases (e.g. hepatitis, HIV) that reduce the risk of transmission to others;
- contraception options that improve compliance and reduce the risk of sexually transmitted infection;
- vaccination that reduces risk of disease in the community.

Along with a narrative on the health benefits of a pharmaceutical to the family, whanau, and wider society; information can be provided on the quality-adjusted life year (QALY) gains of the proposal. If the measure is not the EQ-5D, please provide a mapping of the measure to the EQ-5D, with details on the methodology used for mapping.

For further information on how Pharmac measures and considers health benefits to the family, whānau, and wider society, please refer to the Factors For Consideration.

Question 4: If the proposed pharmaceutical was funded, what would the consequences to the health system be?

As part of making its decision, Pharmac thinks about the consequences that funding a treatment could have on the wider health system[1]. For example, we need to understand if funding a pharmaceutical for a community use would help prevent hospitalisations, or we need to understand the kind of support and training that may be required to maximise the benefits of a treatment.

Please detail the potential flow on effects that funding the proposed pharmaceutical could have on the health system. Please reference all sources of information (including expert opinion).

Consideration should be given to whether the funding of the pharmaceutical would impact the:

- structure of the health system (e.g. move from hospital service provision to the community, or increase in minor surgery undertaken in primary care);
- efficiency of the health system (e.g. increased/reduced pressure on infusion services or investigations for the selection or monitoring of patients);
- Government's strategic intentions for the health system

[1] For this purpose, health system is defined as Vote Health funding where that funding is enabling the delivery of health services. This includes DHBs (and the services they provide), community pharmacies and General Practices.

Please note that the costs/savings to the health system from funding the pharmaceutical is considered in the Costs and Savings Section.

For further information on how Pharmac measures the consequences to the health system from a funding decision, please refer to the Factors For Consideration.

Question 5: Would funding the pharmaceutical have an effect on the Government's strategic intentions for the health system?

When considering a funding application, Pharmac needs to understand whether funding the pharmaceutical could help support the broader strategic intentions that the Government has identified for the health system. These intentions have been identified in several overarching, strategic documents including the Minister of Health's letter of expectations to Pharmac, the New Zealand Health Strategy, the Healthy Ageing Strategy, the DHB Health Targets for the current year, the current Ministry of Health Statement of Intent, the Medicines New Zealand Strategy and the New Zealand Antimicrobial Resistance Action Plan. The current health priorities can be found here.

Category: Costs and Savings; Subcategory: Price

Question 1: What is the proposed selling price of the pharmaceutical?

Question 2: Per pack of

Please state the proposed selling price of the pharmaceutical (ex-manufacturer, GST exclusive) in New Zealand dollars. We require information on the price that the pharmacy will be charged to purchase the pharmaceutical. This is the price listed in the Pharmaceutical Schedule. Please do not include the effect of any special commercial terms e.g. rebates on the price.

Question 3: What is the supplier's selling prices to wholesalers in other OECD countries?

Please provide the supplier's selling price in other Organisation for Economic Co-operation and Development (OECD) countries of each of the forms of the pharmaceutical that are proposed. Please provide prices in local currencies (excluding local taxes) and provide New Zealand dollar (NZD) equivalents (the exchange rates used must be specified). The OECD countries can be restricted to Australia, Canada, and UK.

If the pharmaceutical is not yet marketed in other jurisdictions, please indicate this in your response.

Question 4: Are there any proposed Special Authority criteria or other funding restrictions that you would like Pharmac to consider?

Please indicate whether you would like Pharmac to consider any restrictions on the funding of the pharmaceutical that would target its use to specific patient populations. Funding restrictions include Special Authority criteria, endorsement criteria or by hospital restriction.

Please indicate the reasoning for this request, and whether assumed restrictions would be in place when provided patient numbers and other calculations.

Question 5: Please attach any proposed commercial terms of listing that you would like Pharmac to consider.

Please provide details on any specific commercial terms of listing that you would like Pharmac to consider. These should be related to the pricing or commercial supply of the pharmaceutical, for example, whether Pharmac should consider a rebate or a period of protected listing.

Please indicate the reasoning for this request and whether it is assumed that these commercial terms of listing would be in place in the calculations provided.

Category: Costs and Savings; Subcategory: Budget impact

An Excel Budget Impact Assessment (BIA) template has been developed for pharmaceutical suppliers to calculate patient numbers and budget impact. These spreadsheets have been designed to provide guidance to suppliers on how to present these calculations, therefore assisting Pharmac staff in validating the estimates. These spreadsheets must describe the methods and assumptions used to generate estimates; references for all sources of data; and include cross-references to data sources.

Question 1: Annual cost (savings) to all relevant budget lines.

Show the costs to different budget streams of the proposal from year of listing (first 12 months is year 1, second 12 months is year 2 etc.). Rebates should be entered as negative values. Values should come from the BIA template which has been filled out.

Question 2: Average cost for a patient for treatment duration (if average treatment duration >12 months then enter cost for 12 months treatment).

Enter cost for a single patient of average duration of treatment. If average duration of treatment is greater than 12 months, then enter cost for 12 months treatment.

Question 3: Please attach the completed BIA template.

The BIA template is available from the Pharmac website here.

Category: Costs and Savings; Subcategory: Health related costs and savings

Question 1: Please detail whether there are there any additional health-related costs or savings to the person receiving treatment that are likely to be incurred if the pharmaceutical is funded.

When we assess an application for funding a medicine, we consider the implications of funding the pharmaceutical and the effect that funding the medicine has on the person receiving the pharmaceutical. This assessment includes reviewing the health-related costs or savings that could be incurred by the person receiving treatment. These costs or savings must be health-related. For each item provide the New Zealand price and estimated resource use.

Examples of costs to the patient that may be incurred as a result of funding a treatment are included in Table 1. For details on how to estimate these costs, please refer to the Cost Resource Manual.

To determine if a cost or saving is health related, please consider whether they are partially subsidised by the health system. For example, if funding a pharmaceutical would result in a reduction of the number of GP visits needed by the person receiving treatment, this would

reduce their health-related costs. This is because the costs that the person must pay in GP fees would reduce.

Table 1: Examples of Costs to Patient, Family, Whānau and Society

Costs to Patient	Specific Costs	Examples of Cost Savings
Pharmacy costs	Pharmacy co-payments Manufacturers surcharge and pharmacy mark-up	Comparator treatment has a patient part-charge
Primary health care	General practice visits (cost to patient)	Significantly fewer GP visits required
Hospital care	Travel and accommodation for hospital visits	Reduced hospital visits
Community-based healthcare services	Ambulance part-charge Residential care Dental care	Significant reduction in ambulance emergency call-outs

The following costs should not be included:

- cost of lost wages, reduced productivity and premature mortality (refer to Prescription For Pharmacoeconomic Analysis for details); and
- cost of individuals privately paying for pharmaceuticals.

Please refer to the Factors for Consideration for further details costs relevant to Pharmac.

Where possible, please ensure that quantitative information is provided. For example, an estimate in the reduction or increase of GP visits that may be required, or the type and diagnostic tests that may be required during treatment for the average patient. If quantitative information is unavailable, please provide a full description of the estimated effects.

Question 2: Please detail whether there are any health-related costs or savings that may be experienced by the family, whānau and wider society of the person receiving the treatment, if the pharmaceutical is funded.

When we assess an application for funding a medicine, we consider the implications that funding the pharmaceutical has on the health-related costs or savings which the family or whānau of the person receiving the pharmaceutical may incur. These costs or savings must be health-related. For each item provide the New Zealand price and estimated resource use.

Examples of costs to the patient, family, whānau and society that may be incurred as a result of funding a treatment are included in Table 1.

To determine if a cost or saving is health related, please consider whether they are partially subsidised by the health system. For example, if a pharmaceutical would reduce the number of hospital visits a person would require, this may reduce the health-related costs to their family if a family member is needed to drive them to hospital, and they are eligible to claim National Travel Assistance, as the cost that a family member would have to pay in car fuel would reduce.

Where possible, please ensure that quantitative information is provided. However, if quantitative information is unavailable, please provide a full description of the estimated effects.

Please refer to the Factors for Consideration for further details costs relevant to Pharmac.

Question 3: Please detail whether there are there any additional costs or savings to the health sector that are likely to be incurred if the pharmaceutical is funded.

Please identify and estimate all additional costs and savings to the health sector that may occur if the pharmaceutical is funded – for each item provide the New Zealand price and estimated resource use.

All costs should be clearly described, and sources of cost data provided.

All price estimates should be obtained from New Zealand. Where feasible, resource use estimates should be based on New Zealand information (e.g. number of GP visits, length of hospital stay, etc.). If New Zealand data is not available, international sources may be used, but should be validated for the New Zealand setting.

Examples of costs to the health sector that may be incurred as a result of funding a treatment are included in Table 2. For details on how to estimate these costs, please refer to the Cost Resource Manual.

Table 2: Examples of Costs to the Health Sector

Costs to Health Sector	Specific Costs	Examples of Cost Savings
Pharmacy costs	Pharmacy handling and service fees Pharmacy margin and pack fee Pharmaceutical compounding	Reduced number of dispensing's. Comparator treatment is unregistered. Comparator pharmaceutical requires compounding.
Primary health care	General practice visits Diagnostic and investigative tests Pathology tests	Fewer tests required to determine patient eligibility.
Hospital care	Admitted care Non-admitted care (specialist appointments and emergency room visits) Travel and accommodation subsidy	Proposed treatment is an oral pharmaceutical and current treatment is an infusion. Fewer inpatient stays (shift to primary care). Shorter recovery time following surgery, allowing earlier discharge.
Community-based healthcare services	Palliative care Residential care In-home nursing and home help Ambulance	Significant delay in need for palliative or residential care. Change in type of residential care required (e.g. rest home care versus private hospital). Significantly improved recovery time following surgery.

The cost of administering a pharmaceutical, such as an intravenous infusion, may involve several components which need to be estimated separately (e.g. nurse/specialist time, outpatient facility cost, post-administration monitoring, etc.). Please refer to the Cost Resource Manual for details on how to estimate the cost of administering treatment.

Category: Economic Analysis; Subcategory: N/A

Question 1: Please attach a report detailing the methodology, inputs, and results of a cost-utility analysis along with a summary of the findings. This analysis should be performed based on the methods outlined in the Prescription for Pharmacoeconomic Analysis.

It is strongly recommended that all applications to Pharmac include an economic analysis demonstrating the cost-effectiveness of the pharmaceutical that is the subject of the Application. Please note that if an economic analysis is not provided, Pharmac will need such information,

and its staff will undertake the analysis independently. This will increase assessment timeframes for the Application.

In most cases the economic analysis should be in the form of a cost-utility analysis (CUA), with benefits measured in terms of quality-adjusted life years (QALYs). In cases where the clinical outcomes of the drug and the comparator have been shown to be equivalent, a cost-minimisation analysis may be appropriate. Other forms of cost-effectiveness or cost-benefit analyses should not be provided to Pharmac.

For details on the methodology to use for CUAs to Pharmac, please refer to the Prescription for Pharmacoeconomic Analysis (PFPA)

For applications being made outside of the PharmConnect system, please ensure the following are included:

- a summary of the methodology and results of the CUA in the main Application;
- a separate report detailing the methodology, inputs and results of the CUA (attached to the Application);
- an electronic copy of the model in TreeAgeTM and/or Microsoft Excel (this must be fully transparent (i.e. no hidden cells or 'black boxes') and able to be fully amended).

If an economic analysis has been submitted to the Pharmaceutical Benefits Advisory Committee (PBAC) in Australia, Pharmac will accept the same analysis in the Application to Pharmac, providing an electronic copy of the TreeAgeTM model and/or Excel spreadsheet is included. Please ensure costs (and any other relevant inputs) in the model are amended so they are applicable to the New Zealand clinical and funding environment.

Question 2: Please attach an electronic copy of the cost-utility analysis model in TreeAgeTM and/or Microsoft Excel.

The model must be fully transparent (i.e. no hidden cells or 'black boxes') and able to be fully amended.

If an economic analysis has been submitted to the Pharmaceutical Benefits Advisory Committee (PBAC) in Australia, Pharmac will accept the same analysis in the Application to Pharmac, providing an electronic copy of the TreeAgeTM model and/or Excel spreadsheet is included. Please ensure costs (and any other relevant inputs) in the model are amended so they are applicable to the New Zealand clinical and funding environment.

Question 3: What is the base case point estimate of cost-effectiveness, in QALYs gained per $1 million invested?

Please provide a base case estimate of the cost-effectiveness of the proposed treatment in quality-adjusted life years (QALYs) gained per NZ$1 million.

This estimate may be greater than 0 (i.e. additional cost for additional QALYs) and expressed numerically, or it may be cost-saving (written as 'cost-saving').

For further information please refer to Pharmac's Prescription for Pharmacoeconomic Analysis which can be found on the Pharmac website here.

Question 4: What is the upper limit of the likely range of cost-effectiveness in QALYs per $million?

Please provide the upper limit of the estimated likely range of cost effectiveness of the proposed treatment. The upper limit of the likely range is the maximum likely QALYs gained per NZ$1 million invested from funding the proposal. This result is generated when varying inputs over plausible ranges. For further information please refer to Pharmac's Prescription for Pharmacoeconomic Analysis which can be found on the Pharmac website here.

Question 5: What is the lower limit of the likely range of cost-effectiveness in QALYs per $million?

Please provide the lower limit of the estimated likely range of cost effectiveness of the proposed treatment. The lower limit of the likely range is the minimum likely QALYs gained per NZ$1 million invested from funding the proposal. This result is generated when varying inputs over plausible ranges. For further information please refer to Pharmac's Prescription for Pharmacoeconomic Analysis which can be found on the Pharmac website here.

Category: Suitability; Subcategory: Features of the pharmaceutical that impact its use

Question 1: Are there any features of the pharmaceutical that may impact use by the person receiving the treatment? If so, please explain.

Please provide information on the features of the pharmaceutical that may have an impact on use by the person receiving the treatment, and the outcome of treatment.

Features are particularly relevant if they affect adherence. Examples of features of a pharmaceutical that may impact on use by the patient include (but are not limited to):

- size
- shape
- taste
- coating
- method of delivery (e.g. oral, infusion, etc.)
- ease of use
- time required to administer
- frequency of administration (e.g. once daily versus multiple times per day)
- packaging
- supporting information
- training.

If possible, please provide information on the likely magnitude of impact on the outcome of treatment.

Consideration should be given to whether there are any subgroups in the target population who are more impacted by the features of the pharmaceutical (for example, elderly, children, people with poor dexterity, poor vision, intellectual impairment, etc.).

Please reference all sources of information.

Question 2: What features of the pharmaceutical may have an impact on use by the family or whānau of the person receiving the pharmaceutical, or on wider society?

In cases where the pharmaceutical needs to be administered by someone other than the patient or health workers, please provide information on the features of the pharmaceutical that may impact on use by the family or whānau of the person receiving the pharmaceutical, or on wider society.

Features are particularly relevant if they may impact on adherence and health outcomes of the patient.

The features identified may influence things such as how difficult the pharmaceutical is to administer, the time it takes to administer treatment, or how much dexterity is required to administer treatment (especially for older caregiver partners). Please note that these examples are not exhaustive.

If possible, please provide information on the likely magnitude of impact on the outcome of treatment.

Please reference all sources of information.

Question 3: What features of the pharmaceutical may have an impact on use by the health workforce?

In cases where the pharmaceutical needs to be administered by members of the health workforce, please provide information on the features of the pharmaceutical that may affect use by the health workforce. This includes features which:

- affect how easy it is for a health worker to use,
- affect how likely it is a health worker may make (or prevent) an error
- dissuade workers from using a product at all even though it could be clinically beneficial.

Features particularly relevant to the health workforce could include (but are not limited to):

- training in use,
- confusion with similar products,
- ease of obtaining patient cooperation,
- packaging, and
- supporting information.

Please reference all sources of information.

Question 4: Are there any other issues or benefits that may arise as a result of the features of the pharmaceutical that have not been covered elsewhere in this section?

Please detail any features of the pharmaceutical that may influence the use and outcomes achieved by the pharmaceutical that have not been covered elsewhere in this section.

Category: Additional Information; Subcategory: N/A

Question 1: Please provide any additional information that is relevant to your application.

Question 2: Please attach any additional files that are relevant to your application.

Please provide any further additional information and attachments that have not been provided elsewhere in this application which you consider relevant to your application. For example: review articles, published critiques, international guidance and recommendations, published cost-utility analyses, and expert opinion and consensus reports from expert panels.

Category: Declaration and Identification; Subcategory: Declaration

Question 1: Do you have the right to supply the product for which funding is requested?

You must be able to enter into contract for supply with Pharmac. If not, we cannot consider your application.

Question 2: I confirm that the company I represent has legal rights to the patents

Question 3: I confirm that there are no non-patent intellectual property barriers

This requires confirmation that there are no non-patent IP barriers to supply by the supplier. Examples of non-patent IP barriers include trademarks, design rights, or copyright.

Question 4: I have read and accept Pharmac's standard terms of listing on the Pharmaceutical Schedule.

The General Terms of Listing in Section B of the Pharmaceutical Schedule and General Terms of Listing in Section H of the Pharmaceutical Schedule are the standard clauses within a Pharmac contract.

Question 5: Any variations on the standard terms of listing for Pharmac to consider have been detailed in this Application or provided within an attachment

Question 6: I declare that all known published and unpublished clinical trials relevant to this Application have been disclosed in the Application

Question 7: I declare that all known published and unpublished clinical trials that I am aware of that are relevant to this application have been disclosed in the Application

Question 8: I declare that I have obtained the appropriate permission or paid the appropriate copyright fee for any publication or other information provided in support of this application, and that the publications can be distributed internally by Pharmac (including to Pharmac committees) for the purpose of reviewing the application.

Pharmac requires the ability to use and distribute copies of any publications submitted with the application, for the purpose of reviewing the application. This may include distribution to members of Pharmac's clinical advisory committees. It is the responsibility of the supplier to ensure all required copyright licences and approvals have been obtained.

Question 9: I agree that the product details information provided in the on-line form can be made publicly available on the Application Tracker

Question 10: I confirm the information provided in this Application is correct

Question 11: Do you have any comments regarding any of the above declarations?

Category: Declaration and Identification; Subcategory: Identification

Question 1: Name of person submitting application

Question 2: Date of application

Question 3: Who is the primary contact for this application?

Question 4: What is the primary contact's job title?

Question 5: What is the primary contact's email address?

Question 6: What is the primary contact's phone number?

Information requests for specific products

Vaccines

This section of the Guidelines applies to applications for the listing of a vaccination in the National Immunisation Schedule (section I of the Pharmaceutical Schedule).

Note that applications for the listing of a vaccine must comply with the information requested in Section 1-6 of the Application Guidelines, as applicable. The information requested in this section (Section 7.1) is to be provided in addition to the information requested in each of the prior sections.

Category: Vaccines; Subcategory: Pharmacological Information

Question 1: For the proposed vaccine, please specify the number, identification and amounts of antigens (components)?

Question 2: What is the formulation of the vaccine?

Question 3: What is the nature of the immunising agent(s)?

Please provide details on the nature of the immunising agent, for example, is it live, attenuated or killed; absorbed or non-absorbed; viral or bacterial etc.

Question 4: How is the vaccine presented?

Question 5: What are the external dimensions (measurements) of the vaccine when it is packed for storage?

Question 6: Are there any requirements for cold chain management? Please specify

Category: Vaccines; Subcategory: Proposed amendments to the Pharmaceutical Schedule

Question 1: Is this a new vaccine or an alternative to a vaccine that is currently included in the National Immunisation Schedule?

Question 2: What is the proposed schedule of administration of the vaccine?

Please include details of dose for each of the age or population group, and whether primary immunisation and/or booster vaccinations are requested.

Question 3: Please provide details on whether there are any programme requirements for administration of the vaccine and the potential effects that this would have on the various immunisation providers.

Question 4: Can a vaccination course that begins with the proposed vaccine be completed with a competing or alternative vaccine (or vice versa)? If so, please provide details.

Question 5: Is there any expectation of a limited initial supply?

Question 6: Is a catch-up programme required? If so, please provide details.

A catch-up programme provides coverage of individuals who could benefit from vaccination at the introduction of a new programme, but who are older than the age range specified for

delivery of the ongoing primary vaccination program. A catch-up programme might also provide a faster onset of any herd immunity generated by the vaccine.

If a catch-up programme is also requested, define and justify its duration, and its extent in terms of the additional targeted population groups.

Describe the arrangements for any requested catch-up programme(s) and compare them with those of the requested ongoing primary vaccination programme.

Category: Vaccines; Subcategory: Patient Population

Question 1: In addition to describing the characteristics of the target population, please justify the selection of specific characteristics of the target population, for example the requested age range(s) of eligible individuals within the primary immunisation programme and catch-up programme (if relevant).

Category: Vaccines; Subcategory: Current Treatment

Question 1: Is an alternative vaccine listed on the National Immunisation Schedule?

If an alternative vaccine is listed on the National Immunisation Schedule, this will usually be the main comparator. If there is currently no vaccine available, the main comparator would usually be standard medical management.

Question 2: If there is an alternative vaccine currently on the National Immunisation Schedule, please provide a comparison of the proposed vaccine and the alternative. This comparison should include information on the content and characteristics of the vaccines.

Where the main comparator is an alternative vaccine, present a table to help compare the content and characteristics of the vaccines (eg the antigens included in the vaccines, the strength of the vaccines, the scheduling of doses, the routes of administration, the fit with the current immunisation schedule).

Explain the relationship between the proposed vaccine and vaccines currently available on the National Immunisation Schedule in terms of their antigen content and their dosage schedules. Also address the impact on vaccine efficacy/effectiveness and/or safety arising from co-administration with other vaccines, if relevant.

Category: Vaccines; Subcategory: Health Benefits

Question 1: Please provide evidence of the effectiveness of the vaccine for individuals in the primary and catch-up populations.

Present evidence of the effectiveness of the vaccine for individuals in the primary and catch-up populations.

Ensure that an assessment of comparative harms extends beyond those temporarily associated with the administration of the vaccine to those that might emerge sometime after the vaccine course is completed. This might include the consequences of possibly delaying, rather than preventing, disease because of changes in disease epidemiology and individual susceptibility at a population level at a certain time – including the risks that such delays may generate greater disease burden through greater numbers of susceptible people and/or worse disease severity.

Question 2: Please indicate whether funding the vaccine is likely to provide indirect protection to non-immunised people through appropriate coverage (i.e. herd immunity). Where relevant, please ensure references and evidence that demonstrates indirect protection is provided.

All applications should provide evidence for any health benefits accruing to people other than the person being treated. For preventive vaccines, a key source of such benefits is herd immunity.

Provide evidence that indicates whether funding the vaccine is likely to provide indirect protection to non-immunised people through appropriate coverage (ie. herd immunity). Relevant evidence supporting herd immunity benefits may include:

- The proposed vaccine protects against a new infection/disease and/or reactivation of an existing infectious pathogen to cause disease.
- The efficacy of the proposed vaccine is sufficient to reduce the proportion of susceptible individuals, carriage of the relevant pathogen and/or transmission of the pathogen to susceptible nonimmunised individuals.
- The disease is sufficiently severe or prevalent in an unimmunised population to justify maximising the use of the proposed vaccine to achieve a broader population health benefit.

Special Foods

This section of the Guidelines applies to applications for the listing of a Special Food in Section D of the Pharmaceutical Schedule.

Note that applications for the listing of a Special Food must comply with the information requested in Section 1-6 of the Application Guidelines, as applicable. The information requested in this section (Section 7.2) is to be provided in addition to the information requested in each of the prior sections.

Category: Special Foods; Subcategory: Pharmacological Information

Question 1: Please provide information about all the ingredients in the proposed product. In the case of products that will be used to treat allergies or food intolerances, also include information on the origin of the ingredients.

Question 2: Attach a table on the micronutrient and macronutrient content of the product per 100 kcal, and per 100 g or 100 mL

Question 3: Select type of product

Question 4: If other, please specify

Question 5: Please indicate whether the proposed product is intended to supply all of the protein, energy, fatty acid, vitamin and mineral requirements for a patient if used as a sole source of nutrition. If this is the case, please then identify if there are situations where additional nutritional supplementation may be required (e.g. for catch-up growth in children, other necessary ingredients to meet nutritional needs).

Question 6: If the product is intended to be administered via an enteral pump, please provide details on the products compatibility with currently available medical devices and consumables in New Zealand.

Question 7: If the product is an infant formula, please attach a table comparing the proposed product with the requirements of the Australia New Zealand Food Standards Code – Standard 2.9.1: Infant Formula Products. This table should indicate whether the proposed product complies with the code or justify any deviations from parts of the code. In addition, please provide a further table comparing the proposed product with similar currently listed products for the features that are relevant to the intended indication. For example, Dalton size of the protein.

Category: Special Foods; Subcategory: Regulatory status of product

Question 1: The Australia New Zealand Food Standards Code – Standard 2.9.5: Food for Special Medical Purposes, sets out the requirements for foods that have medical purposes. Please confirm that these requirements have been met.

Category: Special Foods; Subcategory: Proposed amendments to the Pharmaceutical Schedule

Question 1: Please attach a table that provides a comparison of the proposed product and its main comparator products with the nutrient reference values for Australia and New Zealand.

The Nutrient reference values for Australia and New Zealand provide the recommended daily intake reference values that are used in New Zealand. Please attach a table that provides a comparison of the proposed product and its main comparator products with the nutrient reference values for Australia and New Zealand. This table should indicate whether the products provide the required amount of key nutrients for patients for whom the proposed product is intended. Include the following age ranges, as applicable:

infants younger than one year

- children 1–2 years
- children >2–5 years
- children >5–10 years
- older children >10–15 years
- adolescents >15–20 years
- adults >20 years.

Use the midpoint of the age range. For the non-adult age ranges, compare the nutrient calculations for a child whose weight is on the 50th percentile for weight, using the New Zealand Well Child / Tamariki Ora growth charts. For the adult age range, include pregnancy and lactation tables for the product, unless the product is unsuitable for pregnant or lactating women.

Question 2: Please provide the instructions for preparation and use of the proposed product. Include information on the percent solution (weight per volume), the scoop volumetric size and the weight of product it holds, and scoops to water volume for a 'normal' dilution. In addition, please provide the osmolality of the 'normal' dilution.

Community Medical Devices

This section of the Guidelines applies to applications for the listing of a community medical device in the Pharmaceutical Schedule. The Medicines Act 1981 provides the legal definition of a medical device.

Note that this section does not apply to medical devices for use within hospitals. Pharmac is in the relatively early stages of its hospital medical devices work and does not currently require suppliers of hospital medical devices to submit Applications. If unsure whether the pharmaceutical or device is within the scope of Pharmac's activity (eg. if a pharmaceutical has characteristics of a medicine and a device), please contact Pharmac for advice.

A number of medical devices are listed in Section B of the Pharmaceutical Schedule for use in the community. These include (but are not limited to) respiratory devices such as peak flow meters and spacers, condoms, contraceptive copper intrauterine devices, diabetes blood glucose meters and test strips, and insulin pumps and consumables.

Note that applications for the listing of a community medical device must comply with the information requested in Section 1-6 of the Application Guidelines, as applicable. The information requested in this section (Section 7.3) is to be provided in addition to the information requested in each of the prior sections.

Category: Community Medical Devices; Subcategory: Device information

Question 1: Please provide a description of the therapeutic purpose of the device.

Please refer to the Medsafe website for the definition of therapeutic purpose and details on how it applies to medical devices

Question 2: Please provide details of the pack contents and whether any accessories are included.

Question 3: Please describe how the device is used.

Question 4: Please attach the instructions for use and/or the user guide.

Question 5: Does the device need to be used with a pharmaceutical or other technology? If so, is the pharmaceutical or technology is available and funded in New Zealand?

Question 6: What is the lifespan of the device, and of any relevant component parts? Please detail the assumptions that have been made regarding frequency of usage when calculating these lifespans.

Question 7: Are there any different models or versions of the device that are available? Does this have any consequences on the mode of action?

Question 8: What properties or features of the device make it innovative or a significant modification when compared with other technologies of its type?

Category: Community Medical Devices; Subcategory: Regulatory status of the device

Question 1: WAND registration number

In order for medical devices to be legally supplied in New Zealand they must be notified to the WAND database.

Question 2: Date of registration on the WAND database

Category: Community Medical Devices; Subcategory: Proposed amendments to the Pharmaceutical Schedule

Question 1: If applicable, please describe how the device connects with or demonstrates interoperability with current systems used within New Zealand (e-prescribing, e-health records, is it Bluetooth enabled etc).

Further information on Costs and Savings

The following section provides more information on the information that is requested within the Budget Impact Assessment (BIA) template and the Costs and Savings section of the Application form.

Uptake of Pharmaceutical

How many people are likely to be eligible for treatment? How many units are likely to be dispensed?

There are two approaches that can be used to estimate number of patients and uptake of a proposed pharmaceutical – an epidemiological approach and a market-share approach. All estimates should be provided for the first five years of the proposal, shown on an annual basis.

Epidemiological approach

An epidemiological approach estimates the number of people with the medical condition, and then estimates the use of the proposed pharmaceutical.

Please provide the following estimates to include in Table 3 and Table 4 below:

(i) Number of patients with the medical condition

Estimate the likely number of patients with the condition per annum for the first five years of listing, using incidence and/or prevalence data, and accounting for changes in disease or condition incidence or prevalence trends.

Present the methods and assumptions for converting incidence and/or prevalence data to number of patients, including sources of information. If New Zealand specific reference data is available, this must be used. If New Zealand specific data is not available, discuss the applicability of the data used to the New Zealand setting.

Sources of incidence and prevalence data include: Medline; Medscape; UpToDate; Ministry of Health (Mortality Records, New Zealand Health Survey, Hospital Event data); Global Burden of Disease (GBD) study; World Health Organisation (WHO).

The choice to use incidence or prevalence data depends on several factors, including the nature of the medical condition, its treatment and the available data. In general, treatments of short duration are best suited to incidence estimates, and long-term treatments (eg for chronic diseases or conditions) may be better suited to prevalence estimates. A combination of prevalence and incidence estimates may be required.

(ii) Number of patients eligible for the requested restriction

Using the annual numbers of patients with the medical condition for five years, estimate the proportions of patients who would be expected to be eligible for treatment according the proposed Special Authority criteria. If there is uncertainty regarding use within the proposed Special Authority restriction, this should be addressed in the analysis.

The actual number of patients treated may be significantly different from the eligible patient population due to differential response rates to treatment, treatment related adverse events or any other factors (e.g. treatment form, frequency or administration method) impacting treatment

adherence, and slippage (i.e. inappropriate usage or usage beyond the restriction, including indication creep). Where patient numbers are likely to be materially impacted by these considerations, these should be taken into account in a separate analysis.

(iii) Number of patients likely to take the pharmaceutical for the proposed indication

Using the annual numbers of eligible patients, estimate the proportions likely to take the proposed medicine in each of the five years. Ensure that the estimates reflect the rate of uptake of the proposed pharmaceutical and include the impact on the use of other pharmaceuticals. Justify the estimate of uptake and assess variations to this estimate.

(iv) Units dispensed

The estimate of the units dispensed for each of the five years should account for:

- the rate of uptake of the proposed medicine across the five years from listing (as estimated above);
- the dose, frequency and duration of treatment involving the proposed pharmaceutical; and
- different forms and strengths of the proposed medicine.

Present each of the steps for estimating the units dispensed separately.

Ensure that the estimates reflect the quantities of pharmaceutical dispensed, rather than the quantities of medicine consumed, which may be affected by compliance, dose reductions, discontinuations and wastage.

Table 3: Estimate of Incidence and Prevalence

	Year 0	Year 1	Year 2	Year 3	Year 4
New Zealand Population (#)					
Incidence Rate (%)					
Incidence X:100,000					
Incidence (#)					
Prevalent population (#)					
Patients with the condition (#)					
Eligible patients (%)					
Eligible patients (#)					
Patients likely to take treatment (%)					
Patients likely to take treatment (#)					
Adapted from Adapted from Guidelines for preparing a submission to the Pharmaceutical Benefits Advisory Committee, Version 5.0, September 2016 https://pbac.pbs.gov.au/					

Table 4: Units dispensed for each pharmaceutical form

	Year 0	Year 1	Year 2	Year 3	Year 4
Estimated number of patients likely to take treatment (i.e. uptake of pharmaceutical)					
Estimated number of units dispensed					
Adapted from Guidelines for preparing a submission to the Pharmaceutical Benefits Advisory Committee, Version 5.0, September 2016 https://pbac.pbs.gov.au/					

Market-share approach

The market-share approach estimates the extent of the current market represented by the proposed patient indication and, consequently, the share likely to be taken by the proposed medicine. It is likely to be the most suitable approach where a medicine will completely substitute existing pharmaceutical(s).

Please provide the following estimates to include in Table 5 below:

(i) Units dispensed for currently listed pharmaceuticals

Identify the currently available pharmaceuticals that are likely to be substituted by the proposed pharmaceutical in the community and/or hospital.

Estimate the units dispensed in the most recent 12 months of the relevant market. This should be based on medicine utilisation data or studies for currently available medicines that are likely to be substituted by the proposed pharmaceutical.

Script volume data is available at DataPharm.

Where possible, present the units dispensed and the number of patients this represents. Consider the impact of wastage, discontinuations and noncompliance when back-calculating the number of patients from units dispensed, or justify when these factors are unlikely to be important. However, if the duration of therapy or the units dispensed per patient per course of treatment is uncertain, do not back-calculate to patients, as it can introduce significant errors into the patient numbers.

(ii) Rate of market growth for currently listed pharmaceuticals

Estimate the rate of growth in the market of currently available pharmaceuticals over five years following listing. Base this on historical trends in the market or other influences. Justify the estimate of market growth in the absence of the listing of the proposed medicine.

Where more than one pharmaceutical is likely to be substituted, present the market share and rate of growth for each item, if required. Disaggregating the estimated growth according to each pharmaceutical is important if they are likely to have different rates of growth, are likely to be substituted differentially by the proposed pharmaceutical, or have a different cost.

Adjust script volume for the proportion of use applicable to the relevant indication.

(iii) Market share

Estimate the rate of substitution in the market by the proposed medicine for each year over five years (i.e. the proportion of current treatment displaced by the proposed pharmaceutical) . Provide evidence, such as market uptake rates from other markets and the applicability of these markets to the New Zealand setting, to justify the estimate of market share. Clearly

communicate and justify the likely extent of market uptake following listing of the proposed medicine.

Present the estimate of the rate of substitution for each of the following, if required:

- different pharmaceuticals that will be substituted where the rate of growth is different, the rate of substitution is different or the cost is different
- different forms, doses and durations of treatment.

(iv) Growth of the market after listing

Growth in the market can come from a range of sources including marketing; delays in disease progression; intolerance or treatment failure with current treatment; and changes in patient or health professional preference.

Estimate the units dispensed for the proposed pharmaceutical for each year that is above the growth projected in the market using historical data. Report both the expected increase in patient numbers, and the expected units for each form, strength and duration for the proposed medicine. Justify when no additional growth in the market is predicted.

Provide references to data of similar circumstances in similar markets, and discuss risks associated with market growth, to increase the certainty of the financial implications of listing the proposed medicine.

Table 5

	Year 1	**Year 2**	**Year 3**	**Year 4**	**Year 5**
Estimated script volume of currently listed pharmaceuticals					
Estimated annual rate of growth for currently listed pharmaceuticals					
Rate of substitution in market					
Estimated units dispensed above growth in market					
Net units dispensed					
Adapted from Guidelines for preparing a submission to the Pharmaceutical Benefits Advisory Committee, Version 5.0, September 2016 https://pbac.pbs.gov.au/					

Budget Impact

What is the gross estimated budget impact? What impact will listing the pharmaceutical have on the use of other pharmaceuticals? What is the net pharmaceutical budget impact?

An Excel BIA template has been developed for Pharmaceutical Suppliers to calculate patient numbers and budget impact. These spreadsheets have been designed to provide guidance to Suppliers on how to present these calculations, therefore assisting Pharmac staff in validating the estimates. These spreadsheets must describe the methods and assumptions used to generate estimates; references all sources of data; and include cross-references to data sources.

Gross Budget Impact

Gross budget impact refers to the financial impact of listing the pharmaceuticals without taking into account the offsets of substituted pharmaceuticals.

Present in a table the total estimated budget impact for each of the forms and strengths of the proposed pharmaceutical over 5 years following listing. State whether funding would affect the community pharmaceutical budget and/or hospital pharmaceutical budget.

Impact on Use of Other Pharmaceuticals

Pharmaceuticals likely to be affected by the listing of the proposed medicine include:

(i) pharmaceuticals substituted by the proposed pharmaceuticals;
(ii) supplementary pharmaceuticals with decreased usage (e.g. pharmaceuticals co-administered with the substituted pharmaceuticals or used to treat clinically significant adverse reactions to the substituted pharmaceuticals)
(iii) supplementary pharmaceuticals with increased usage (e.g. pharmaceuticals co-administered with the proposed pharmaceutical or used to treat clinically significant adverse reactions to the proposed pharmaceutical)

List all pharmaceuticals that fall into each of these three categories (all forms and strengths); estimate the utilisation change; and calculate the financial impact over five years. Reference how the estimates were generated and the data on which the estimates were based.

Net Budget Impact

Present the net budget implications over five years, accounting for the estimated cost of the proposed pharmaceutical; the increased usage of other pharmaceuticals; and cost offsets for substituted medicines with a likely reduction in usage. This is illustrated in the Table 6.

Table 6: Net Budget Impact

	Year 1	**Year 2**	**Year 3**	**Year 4**	**Year 5**
Proposed pharmaceutical cost					
Plus supplementary pharmaceuticals with increased usage					
Less substituted pharmaceutical cost per patient					
Less supplementary pharmaceuticals with decreased usage					
NET BUDGET IMPACT					

Net Budget Impact to the Health Sector

Please provide the following information:

(ii) net budget impact to the health sector.

Please provide the following information:

(i) net budget impact to the health sector over 5 years following listing (excluding pharmaceutical costs);

(ii) net budget impact to the health sector over 5 years following listing (including pharmaceutical costs).

The costs included in these estimates should be specified.

Part 3: Background information and Frequently Asked Questions

Background Information and Frequently Asked Questions

Pharmac – an introduction

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.

What are the Factors for Consideration?

The Factors for Consideration are the framework Pharmac uses when making funding decisions. For more information about the Factors, see https://www.pharmac.govt.nz/medicines/how-medicines-are-funded/factors-for-consideration/

What is a funding Application?

A funding Application is a proposal made to Pharmac to amend the Pharmaceutical Schedule. This is done through either the funding of a new pharmaceutical; or by making changes to the funding criteria for a pharmaceutical that is already on the Pharmaceutical Schedule (for example, a proposal to widen or restrict access).

Who can make a funding Application?

There are no restrictions on who can make an Application to Pharmac. Funding applications can be made by pharmaceutical suppliers, health professionals, clinical groups, consumer groups, members of the general public, Pharmac staff and clinical advisors and DHB staff.

How do I make a funding Application to Pharmac ?

Pharmac requests that funding Applications are made online using Pharmac's PharmConnect system. The PharmConnect system can be accessed from the Pharmac website. Separate application forms are available on PharmConnect for suppliers, clinicians and members of the general public.

For more information on the PharmConnect system, please visit the Pharmac website.

What information will I need to provide in a funding Application?

The Application forms within the PharmConnect system consist of a series of questions. A list of these questions can be found in Appendix 1. Further details on the information we are seeking from these questions has been provided within this document

In answering these questions, we anticipate that, in the majority of cases, enough information will be provided to allow us to initiate assessment of an Application. However, it is important to note that these questions are not exhaustive or prescriptive, and more or less information may be required depending on the circumstances of the funding Application.

I need more information on making a funding Application, who should I contact?

Clinicians and members of the general public who wish to make a funding application should call our freephone information line on 0800 660 050 (9am to 5pm weekdays, calling from within New Zealand) or email our enquiries team at enquiry@pharmac.govt.nz. They will be able to assist with your questions.

Pharmaceutical suppliers should call Pharmac on +64 4 460 4990 before making an Application for a proposed amendment to the Pharmaceutical Schedule. 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.

Does the pharmaceutical need to be registered by Medsafe?

We consider funding applications relating to medicines that have been either Medsafe registered, or submitted to Medsafe for funding. Funding applications for unregistered medicines are typically only considered on a case-by-case basis.

Unregistered medicines

For the medicine or indication to be considered by Pharmac for funding in parallel with Medsafe assessment, there needs to be an active application lodged with Medsafe and evidence of payment of Medsafe application fees should be provided.

Medicines for rare disorders

Suppliers of medicines for rare disorders do not need to have submitted applications to Medsafe for regulatory approval before applying to Pharmac , but the pharmaceutical must be Medsafe-approved before a final decision can be made to fund it. Funding applications for medicines for rare disorders, where Medsafe approval or submission has not yet occurred, need to meet the following three policy principles:

1. Medsafe, or an approved international regulatory authority, has approved the medicine for the specific indication or condition.
2. The disorder is a clinically defined disorder that affects an identifiable and measurable patient population of less than 1:50,000 in New Zealand.
3. The medicine is only registered to treat the rare disorder. If it is registered for other disorders (or is part of phase three clinical trials for other disorders), those other disorders must meet principle 2.

Does the supplier need to hold the rights to supply the pharmaceutical?

Yes, in order to submit a proposal to Pharmac , the supplier must hold the rights to supply the pharmaceutical. Suppliers must be able to enter into a contract for supply with Pharmac. If not, we cannot consider the Application.

Is the information requested mandatory to provide?

The information requested in these Guidelines is the information Pharmac generally requires in order assess a proposal and compare it with other funding options. Please note that Pharmac's on-line application form in the PharmConnect system does have some mandatory fields for information Pharmac considers to be essential to undertake an assessment. It is not possible to submit an Application via the PharmConnect system unless these fields are completed.

Any information that is not available or is otherwise not supplied, must be stated explicitly within the Application and an explanation of its absence should be provided.

If information is not provided that Pharmac requires, it is likely that Pharmac will either contact you for the information or undertake its own searches or analysis (which may result in time delays). Therefore, even though it is possible to submit an application that does not contain all of the requested information, we strongly recommend that you answer all questions thoroughly and we note that there are significant timeliness advantages in doing this.

However, please be aware that these Guidelines are not prescriptive. We acknowledge that there may be situations where less information is available on a pharmaceutical, and that these Applications may be more succinct.

In all instances we strongly recommend you contact Pharmac for guidance on the information required for your particular proposal.

How do I make an application for a generic or biosimilar pharmaceutical?

If you wish to submit a funding application for a generic or biosimilar pharmaceutical, please contact the relevant Therapeutic Group Manager to discuss your application.

How do I submit a funding Application outside of the PharmConnect system?

Pharmac strongly encourages applicants to make funding applications using the online PharmConnect system. If you are unable to submit an Application using the PharmConnect system, please contact Pharmac to discuss your needs. Clinicians and members of the general public who wish to make a funding application should call our freephone information line on 0800 660 050 (9am to 5pm weekdays, calling from within New Zealand) or email our enquiries team at enquiry@pharmac.govt.nz. Pharmaceutical suppliers should call Pharmac on +64 4 460 4990.

Is there anything else I should be aware of in relation to the evidence I need to provide with my Application?

If including unpublished articles, specify why each trial has not been published and their expected dates of publication (if applicable).

Abstracts and posters are not usually appropriate sources for descriptions of the study methodology or primary outcomes of studies. However, if they are adequately detailed, they may be used as references to update information after the primary analyses or any analyses of secondary outcomes not detailed in the published report.

Articles Not to Attach to Applications

Please do not attach copies of the following:

- phase 1 clinical trials;
- studies that do not meet the inclusion criteria;
- narrative reviews;
- reviews of the disease;
- studies outlining the biological rationale for the treatment;
- information unrelated to the pharmaceutical and disease under consideration;
- expert opinion.

How should the references be cited?

All evidence cited should be referenced appropriately, and should be numbered in the order in which they first appear in the text. All references should be listed within the requested citation list in the Health Benefits section in the Vancouver style.

Where a question requests that all sources of information are included, it is sufficient to include a reference to the source rather than attach each article. However, if you consider that the article is key to the assessment of the proposal, please include within the attachments.

Would you like samples sent along with my Application?

Applications should contain images that demonstrate the New Zealand packaging that will be used for the pharmaceutical, the labelling of this packaging and images of the pharmaceutical itself. The images should be in the form of suitable graphics, artwork or photographs in pdf or jpeg format. Samples of the New Zealand packaging may also be useful during the evaluation.

This information is used to help assess the pharmaceutical and provides us with information on what support may be required by patients and healthcare staff should the pharmaceutical be subsidised.

In some instances, a sample of the product may be required. If this is the case, the relevant Therapeutic Group Manager will contact you to discuss and arrange a sample to be provided.

How will you manage my Commercial-in-confidence data?

Any information we receive as part of an Application is subject to the Official Information Act 1982 (OIA) and we will consider any request to have information withheld in accordance with our obligations under the OIA. Anyone providing information should be aware the content of the information and identity may need to be disclosed in response to an OIA request.

We are not able to treat any part of your information as confidential unless you specifically request that we do, and then only to the extent permissible under the OIA and other relevant laws and requirements. If you would like us to withhold any commercially sensitive, confidential, proprietary or personal information included in your Application, please clearly state this in your Application and identify the relevant sections of your Application that you would like withheld. Pharmac will give due consideration to any such request.

Appendix 1 - List of Questions

The following is a list of the questions that are on the PharmConnect application form for suppliers:

Category	Subcategories		Questions
Product Overview	Product details	1	Please select the type of application you would like to make.
		2	If other, please specify.
		3	If available, please detail the following codes:
		4	Have any sample(s) of the pharmaceutical been sent to Pharmac ?
		5	If a sample has been sent, please provide information that could help us to manage the sample.
		6	Please attach suitable graphics, artwork or photographs in pdf or jpeg format of the following: The New Zealand packaging of the pharmaceutical, the product itself, and the product labelling.
	Pharmacological information	1	What is the registered name of the generic pharmaceutical?
		2	What is the brand name of the pharmaceutical?
		3	Please provide a brief description of the principal pharmacological action of the pharmaceutical.
		4	Please select the appropriate category for this application.
		5	Please provide information on the various forms, strengths, and pack sizes of the pharmaceutical that you are seeking funding for.
		6	Provide stability data for infusion treatments (if relevant).
	Proposed amendments to the Pharmaceutical Schedule	1	Please provide details on the indications for which funding is sought.
		2	In what setting will the pharmaceutical be used?
		3	Where is the pharmaceutical likely to be used?

Category	Subcategories		Questions
		4	If other, please specify.
		5	Please provide a summary statement of the main therapeutic claims of the pharmaceutical and its proposed use.
	Dose	1	Please provide details on the course of treatment that would be likely used in New Zealand clinical practice for each indication for which funding is requested. This should include both the dose and the duration of treatment.
		2	Is the dosage recommended in the New Zealand data sheet, or the dosage regimen used in the pivotal trials, different from the dosage regimen likely to be used in NZ clinical practice? If so, please provide details.
		3	Do you have any post marketing data on dosage in clinical practice? If so, please provide details.
	Regulatory status of the product	1	Has the pharmaceutical been registered with Medsafe for all indications for which funding is sought?
		2	Please provide details of the Medsafe registered indications
		3	Please attach the Medsafe approved datasheets
		4	Are other formulations of the pharmaceutical registered for use in New Zealand? If so, please provide details
		5	Please provide information on other OECD (Organisation for Economic Co-operation and Development) countries where registration has been sought and approved. In your response, please indicate whether there are any variations in registered indications between New Zealand and the other country and if any boxed warnings are associated with the pharmaceutical
		6	Please provide information on other OECD countries where registration has been sought and declined. Please provide information on the reasons for decline.
		7	Provide details of other presentations or formulations of the pharmaceutical that have been submitted for approval, or are already approved, in other OECD countries.
		8	Are you aware of the pharmaceutical being used to treat any other indications other than the registered indications? If so, please provide details.

Category	Subcategories		Questions
		9	For unregistered products or indications, please provide details of when the applications for registration was filed with Medsafe. If known, please indicate the expected date of registration.
		10	If the Medsafe registration document is unavailable, please attach copies of relevant Food and Drug Administration (FDA) and/or European Medicines Agency (EMA) assessment.
	Patent information	1	Please list all relevant granted and pending patents (in order of relevance) that have claims relating to the pharmaceutical that is the subject of the Application, including the patent number and patent expiry date. These may include chemical, formulation and relevant indication patents.
Health Need	The disease and its impacts	1	Please provide an overview of the disease that would be treated by the proposed pharmaceutical.
		2	How unwell is a person with the disease? Please provide details on the severity of symptoms experienced by the average patient.
		3	Does this disease or condition have an impact on patient health-related quality of life? If so, please provide details on areas of health-related quality of life that are likely to be impacted and severity.
		4	If possible, please provide information on the total undiscounted quality-adjusted life year (QALY) loss associated with the disease (i.e. QALY of patients with the disease compared with the QALY of the same age specific population in perfect health).
		5	Does the disease impact on the health of the patient's family, whānau or wider society? Please explain and provide sources of information.
		6	What is the impact of the disease on Māori health outcomes? Does the disease fall into one of the categories of Pharmac's Māori health areas of focus? Please explain and provide sources of information.
		7	Does the disease disproportionately affect population groups that may already be experiencing a health disparity?
		8	Is the disease a Government health priority?
		9	If yes, please indicate which category the disease falls into.

Category	Subcategories		Questions
	Patient population	1	Who is the target population?
		2	What is the prevalence and incidence of the disease in New Zealand? Please provide estimates of the number of people in New Zealand who have the disease, the number of Māori in New Zealand with the disease and the number of Pacific people in New Zealand with the disease, and how these numbers change over time (this data can be provided in the epidemiology table template). Please also provide details on how these numbers were obtained and how they are applicable to the New Zealand setting.
		3	Please attach the completed Epidemiology Data template.
	Current treatment options	1	What treatments are currently used in New Zealand to treat the disease? Please describe the current treatment algorithm for the target population and if possible, include a flowchart illustrating the current management of the disease in the target New Zealand population.
		2	What sources of evidence were used to inform the current treatment algorithms that you have provided?
		3	Please provide commentary around how well the current treatments work for the disease being treated. In your response, please also provide details of any risks or any tolerability issues associated with the current treatment options.
		4	Are there any issues regarding the availability or suitability of current treatments for this indication?
		5	What is the recommended dose of current treatments and dose equivalencies between current treatment and the proposed pharmaceutical?
		6	What is the shelf life of the current treatment? Please provide information on how this compares to the shelf life of the proposed pharmaceutical.
		7	How would the proposed treatment change the current treatment algorithm? Please include a flowchart illustrating the expected changes in clinical management
Tab	Section		Question

Category	Subcategories		Questions
Health Benefits	Identification and Selection of Studies	1	How was the literature searched? Please provide details on the search strategy that was used to retrieve clinical studies.
		2	What inclusion and exclusion criteria were used in the selection of studies?
		3	Please provide a flow diagram of the number of studies included and excluded at each stage.
		4	What studies met the inclusion criteria?
		5	What studies were identified in the literature search and which were excluded? Please provide a citation list of all identified studies, and indicate which trials were excluded and the basis for their exclusion.
		6	Please attach all identified randomised controlled trials that meet the inclusion criteria.
		7	Please attach all identified meta-analyses and systematic reviews that meet the inclusion criteria.
		8	Please attach all identified high quality cohort studies and case-control studies that meet the inclusion criteria.
		9	Please attach all supplements, errata, editorials, study protocol publications, and journal correspondence relating to the pivotal published trials.
		10	Please attach a register of all ongoing trials that should provide additional evidence in the next 12 months.
	Trial Design and Characteristics	1	Please provide a summary of the methodology for each of the pivotal clinical trials.
		2	Please describe the characteristics of the participants for each of the pivotal trials.
	Trial Results	1	What were the outcomes and methods of analysis in the pivotal trials? For each pivotal trial, please create one record. Please add additional entries as required.
		2	What did the pivotal trials demonstrate? Please provide a summary of the study results for each relevant comparison and outcome.

Category	Subcategories		Questions
		3	Are there any factors that may influence the applicability of clinical study results to patients in routine clinical practice in New Zealand? Would the same clinical benefits and adverse effects be expected?
		4	Please identify, discuss and justify any clinically important differences in the results between the different arms of a trial and between trials.
		5	Does the pharmaceutical have similar, greater, or fewer side effects and/or toxicity when compared to current treatment options? Please provide details.
		6	What impact does the proposed pharmaceutical have on patient-reported outcome measures?
	Interpretation of the Evidence	1	Please provide a general interpretation of the evidence base, considering the clinically significant health benefits and potential health losses to the patient that could be gained from the pharmaceutical, relative to those of the comparator.
		2	If available, please provide details of the incremental health benefits associated with the proposed treatment when compared to the comparator treatment. Where available, this information should be presented in the form of quality-adjusted life year (QALY) gains.
		3	Please provide a general interpretation of the evidence base, considering the clinically significant health benefits and potential health losses that could be gained from the pharmaceutical, to the family and whānau of the person receiving the treatment, and to wider society.
		4	If the proposed pharmaceutical was funded, what would the consequences to the health system be?
		5	Would funding the pharmaceutical have an effect on the Government's strategic intentions for the health system?
Costs and savings	Price	1	What is the proposed selling price of the pharmaceutical?
		2	Per pack of:
		3	What is the supplier's selling prices to wholesalers in other OECD countries?
		4	Are there any proposed Special Authority criteria or other funding restrictions that you would like Pharmac to consider?

Category	Subcategories		Questions
		5	Please attach any proposed commercial terms of listing that you would like Pharmac to consider.
	Budget Impact	1	Annual cost (savings) to all relevant budget lines:
		2	Average cost for a patient for treatment duration (if average treatment duration >12 months then enter cost for 12 months treatment.
		3	Please attach the completed BIA template.
	Health Related Costs and Savings	1	Please detail whether there are there any additional health-related costs or savings to the person receiving treatment that are likely to be incurred if the pharmaceutical is funded.
		2	Please detail whether there are any health-related costs or savings that may be experienced by the family, whānau and wider society of the person receiving the treatment, if the pharmaceutical is funded.
		3	Please detail whether there are there any additional costs or savings to the health sector that are likely to be incurred if the pharmaceutical is funded.
Economic Analysis	N/A	1	Please attach a report detailing the methodology, inputs, and results of a cost-utility analysis along with a summary of the findings. This analysis should be performed based on the methods outlined in the Prescription for Pharmacoeconomic Analysis.
		2	Please attach an electronic copy of the cost-utility analysis model in TreeAgeTM and/or Microsoft Excel.
		3	What is the base case point estimate of cost-effectiveness, in QALYs gained per $1 million invested?
		4	What is the upper limit of the likely range of cost-effectiveness in QALYs per $million?
		5	What is the lower limit of the likely range of cost-effectiveness in QALYs per $million?
Suitability	Features of the pharmaceutical that impact its use	1	Are there any features of the pharmaceutical that may impact use by the person receiving the treatment? If so, please explain.

Category	Subcategories		Questions
		2	Are there any features of the pharmaceutical that may have an impact on its use by the family or whānau of the person receiving the pharmaceutical, or on wider society?
		3	Are there any features of the pharmaceutical that may have an impact on its use by the health workforce?
		4	Are there any other issues or benefits that may arise as a result of the features of the pharmaceutical that have not been covered elsewhere in this section?
Additional Information		1	Please provide any additional information that is relevant to your application.
		2	Please attach any additional files that are relevant to your application.
Declaration and Identification	Declaration	1	Do you have the right to supply the product for which funding is requested?
		2	I confirm that the company I represent has legal rights to the patents
		3	I confirm that there are no non-patent intellectual property barriers
		4	I have read and accept Pharmac's standard terms of listing on the Pharmaceutical Schedule.
		5	Any variations on the standard terms of listing for Pharmac to consider have been detailed in this application or provided within an attachment
		6	I declare that all known published and unpublished clinical trials relevant to this Application have been disclosed in the Application
		7	I declare that all known published and unpublished clinical trials that I am aware of that are relevant to this application have been disclosed in the Application
		8	I declare that I have obtained the appropriate permission or paid the appropriate copyright fee for any publication or other information provided in support of this application, and that the publications can be distributed internally by Pharmac (including to Pharmac committees) for the purpose of reviewing the application.
		9	I agree that the product details information provided in the on-line form can be made publicly available on the Application Tracker
		10	I confirm the information provided in this Application is correct
		11	Do you have any comments regarding any of the above declarations?

Category	Subcategories		Questions
	Identification	1	Name of person submitting application
		2	Date of application
		3	Who is the primary contact for this application?
		4	What is the primary contact's job title?
		5	What is the primary contact's email address?
		6	What is the primary contact's phone number?

Additional Questions Required for Specific Pharmaceuticals

Certain types of pharmaceutical generally require further information as standard to help inform their application. This further information can be obtained from the questions identified below. These questions are listed on PharmConnect and are usually required when making a funding application for a pharmaceutical that falls within one of these categories:

Tab	Section		Question
Vaccines - additional information	Pharmacological Information	1	For the proposed vaccine, please specify the number, identification and amounts of antigens (components)?
		2	What is the formulation of the vaccine?
		3	What is the nature of the immunising agent(s)?
		4	How is the vaccine presented?
		5	What are the external dimensions (measurements) of the vaccine when it is packed for storage?
		6	Are there any requirements for cold chain management? Please specify
	Proposed amendments to the Pharmaceutical Schedule	1	Is this a new vaccine or an alternative to a vaccine that is currently included in the National Immunisation Schedule? Please select

		2	What is the proposed schedule of administration of the vaccine?
		3	Please provide details on whether there are any programme requirements for administration of the vaccine and the potential effects that this would have on the various immunisation providers.
		4	Can a vaccination course that begins with the proposed vaccine be completed with a competing or alternative vaccine (or vice versa)? If so, please provide details.
		5	Is there any expectation of a limited initial supply?
		6	Is a catch-up programme required? If so, please provide details.
	Patient Population	1	In addition to describing the characteristics of the target population, please justify the selection of specific characteristics of the target population, for example the requested age range(s) of eligible individuals within the primary immunisation programme and catch-up programme (if relevant).
	Current Treatment	1	Is an alternative vaccine listed on the National Immunisation Schedule?
		2	If there is an alternative vaccine currently on the National Immunisation Schedule, please provide a comparison of the proposed vaccine and the alternative. This comparison should include information on the content and characteristics of the vaccines.
	Health Benefits	1	Please provide evidence of the effectiveness of the vaccine for individuals in the primary and catch-up populations.
		2	Please indicate whether funding the vaccine is likely to provide indirect protection to non-immunised people through appropriate coverage (i.e. herd immunity). Where relevant, please ensure references and evidence that demonstrates indirect protection is provided.
Special Foods - Additional Information	Pharmacological Information	1	Please provide information about all the ingredients in the proposed product. In the case of products that will be used to treat allergies or food intolerances, also include information on the origin of the ingredients.
		2	Attach a table on the micronutrient and macronutrient content of the product per 100 kcal, and per 100 g or 100 mL
		3	Select type of product

		4	If other, please specify
		5	Please indicate whether the proposed product is intended to supply all of the protein, energy, fatty acid, vitamin and mineral requirements for a patient if used as a sole source of nutrition. If this is the case, please then identify if there are situations where additional nutritional supplementation may be required (e.g. for catch-up growth in children, other necessary ingredients to meet nutritional needs).
		6	If the product is intended to be administered via an enteral pump, please provide details on the products compatibility with currently available medical devices and consumables in New Zealand.
		7	If the product is an infant formula, please attach a table comparing the proposed product with the requirements of the Australia New Zealand Food Standards Code – Standard 2.9.1: Infant Formula Products. This table should indicate whether the proposed product complies with the code or justify any deviations from parts of the code. In addition, please provide a further table comparing the proposed product with similar currently listed products for the features that are relevant to the intended indication. For example, Dalton size of the protein.
	Regulatory status of product	1	The Australia New Zealand Food Standards Code – Standard 2.9.5: Food for Special Medical Purposes, sets out the requirements for foods that have medical purposes. Please confirm that these requirements have been met.
	Proposed amendments to the Pharmaceutical Schedule	1	Please attach a table that provides a comparison of the proposed product and its main comparator products with the nutrient reference values for Australia and New Zealand.
		2	Please provide the instructions for preparation and use of the proposed product. Include information on the percent solution (weight per volume), the scoop volumetric size and the weight of product it holds, and scoops to water volume for a ‘normal’ dilution. In addition, please provide the osmolality of the ‘normal’ dilution.
Community Medical Devices - Additional Information	Device information	1	Please provide a description of the therapeutic purpose of the device.

		2	Please provide details of the pack contents and whether any accessories are included.
		3	Please describe how the device is used.
		4	Please attach the instructions for use and/or the user guide.
		5	Does the device need to be used with a pharmaceutical or other technology? If so, is the pharmaceutical or technology is available and funded in New Zealand?
		6	What is the lifespan of the device, and of any relevant component parts? Please detail the assumptions that have been made regarding frequency of usage when calculating these lifespans.
		7	Are there any different models or versions of the device that are available? Does this have any consequences on the mode of action?
		8	What properties or features of the device make it innovative or a significant modification when compared with other technologies of its type?
	Regulatory status of the device	1	WAND registration number
		2	Date of registration on the WAND database
	Proposed amendments to the Pharmaceutical Schedule	1	If applicable, please describe how the device connects with or demonstrates interoperability with current systems used within New Zealand (e-prescribing, e-health records, is it Bluetooth enabled etc).

Appendix 2 – 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 of 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 (eg 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 (ie outside of the hospital).
Combination product	Products that consist of two or more pharmaceuticals.
Combined Pharmaceutical Budget	The combined pharmaceutical budget, set by the Minister of Health, includes funding for pharmaceuticals used in the community (including vaccines and medical devices), cancer medicines (whether used in hospital or in the community) and some haemophilia treatments. 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.
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).

Term	Definition
Cost-effectiveness analysis (CEA)	Cost-effectiveness analysis (CEA) compares the relative costs of interventions with 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 (ie 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	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. 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 (eg 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.
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 for a treatment (i.e. tightening of access), or cessation of treatment.
Disability-adjusted life year (DALY)	Burden of disease measure that combines years of life lost due to premature mortality and years of life lost due to time lived in states of less than full health.

Term	Definition
District health board (DHB)	The Crown entities responsible for ensuring the provision of publicly funded health and disability support services for the population of a specific geographic area in New Zealand. There are currently 20 DHBs.
Health-related quality of life	Physical, social and emotional aspects of patient's wellbeing.
Hospital medicine	Pharmaceutical that is predominantly administered within the hospital and is funded by DHBs.
Effectiveness	Benefit of treatment in 'real world' setting.
Efficacy	Benefit of treatment in defined population in controlled or ideal circumstances (e.g. randomised controlled trials).
Factors	Factors for Consideration
Gazette notice	Announcement of Medsafe approval for the marketing of a pharmaceutical in New Zealand.
Generic pharmaceutical	A pharmaceutical that contains the same active ingredients as the original branded (and usually patented) formulation. Generic pharmaceuticals are bioequivalent to the branded pharmaceutical with respect to pharmacokinetic and pharmacodynamic properties.
Graphic Appraisal Tool for Epidemiology (GATE)	Tool developed for the critical appraisal of clinical literature.
Incidence	The count of new cases of disease in a defined population during a specified period of time.
Incidence rate	The count of new cases of disease in a defined population within a specified period of time, divided by the number of persons (ie population) at risk (or person-time) of developing the disease during that time period.
Incremental cost	The difference between the cost of an intervention and the cost of the comparator.
Indication	A valid, or generally accepted, use of a medicine.
Marginal cost	The additional cost of one extra unit of product or treating one additional patient.
Markov model	A statistical representation of discrete, recurrent events over time in which the probability of transition from one to another depends on the current state.

Term	Definition
Medicines	Medicines as defined in s.3 Medicines Act 1981.
Medsafe	New Zealand Medicines and Medical Devices Safety Authority.
Medsafe datasheet	Prescribing information provided by the pharmaceutical supplier (and approved by Medsafe) on a specific medicine registered by Medsafe.
Meta-analysis	A systematic process for finding, evaluating and combining the results of data from independent sources.
Monte Carlo simulation	Simulation modelling that uses random numbers to capture effects of uncertainty.
Named Patient Pharmaceutical Assessment (NPPA)	Operating alongside the Schedule, NPPA refers to Pharmac's consideration of applications for individual patients seeking funding approval for treatments not listed on the Schedule, either at all or for that individual patient's clinical circumstances.
Number needed to harm (NNH)	The number of patients who are treated that would lead to one additional person being harmed compared with patients who receive the control treatment. NNH=1/ARI
Number needed to treat (NNT)	The number of patients who need to be treated in order to prevent or create one additional event occurring over a predefined period of time. NNT=1/ARR
OIA	Official Information Act 1982.
Opportunity cost	Value of the alternative options that could be undertaken with the same resources.
Patent	The official document (also known as letters patent) setting out the government's grant of an exclusive right to an inventor to manufacture, use or sell an invention for a certain number of years.
Perspective	Viewpoint of analysis (e.g. funder, society, government, individual).
Pharmaceutical	Pharmaceuticals as defined in s.4 Pae Ora (Healthy Futures) Act 2022.
Pharmaceutical Benefits Advisory Committee (PBAC)	Independent statutory body in Australia that makes recommendations and gives advice to the Australian Minister of Health about which drugs and medicinal preparations should be made available as pharmaceutical benefits.
Pharmaceutical Budget	See *Combined Pharmaceutical Budget.*
Pharmac	The Pharmaceutical Management Agency (Pharmac).

Term	Definition
Pharmaceutical Schedule	The Pharmaceutical Schedule is the list of all the medicines and therapeutic products that District Health Boards (DHBs) fund. The Schedule lists medicines by chemical name (e.g. aspirin) and brand name (e.g. Ethics Aspirin). It lists the formulations, doses and subsidy price of the medicine, as well as any prescribing guidelines or access criteria. The Schedule is divided into nine sections (A-I). It lists medicines that are funded via the Pharmaceutical Budget (community medicines, cancer treatments, vaccines and haemophilia treatments) and those that must be funded by DHB hospitals for use in the hospital (some of which are contracted to be given in public hospitals at specified prices). It also lists more than 6000 hospital medical devices that DHBs may order at a nationally contracted price.
Pharmacology and Therapeutic Advisory Committee (PTAC)	An expert committee of senior health practitioners which provides objective advice to Pharmac on pharmaceuticals and their benefits.
Prescription for Pharmacoeconomic Analysis (PFPA)	The document that provides an overview of Pharmac's cost-utility analysis methodology.
Prevalence	The number of existing cases of disease in a defined population at a notional point in time.
Prevalence rate	The number of existing cases of disease in a defined population at a notional point in time, divided by the number of persons in the population at that time.
PYLL(80)	Potential years of life lost before the age of 80. PYLL measures the time (in years) lost by a population due to premature death. This involves choosing an arbitrary limit to life, so that the duration of life lost due to each death is that potential limit minus the age at death. In the case of PYLL(80), the arbitrary age limit chosen is 80 years. Note that the subtraction is truncated, so that any deaths occurring after people attain that arbitrary age limit have a potential loss of life of zero years.
Quality-adjusted life years (QALY)	A QALY ('quality adjusted life year') is a standard economic measure, which combines the effects of changes in the length and quality of life that result from treatment. Quality-adjusted life years help compare gains in the quality of life with gains in the quantity (length) of life, in a simple and direct manner. Quality of life weightings (or utilities) are typically measured on a scale of 0 to 1, where 0 is equivalent to death and 1 to perfect health. These weights can then be summed over life expectancy in order to calculate the total number of QALYs. The difference in QALYs and overall costs gained between two treatments informs the relative cost-effectiveness of an intervention.
Relative risk	Ratio of incidence of disease in exposed group divided by incidence of disease in non-exposed (control) group.

Term	Definition
Relative Risk Increase (RRI)	Proportional increase in rates of events between the experimental group and control group.
Relative Risk Reduction (RRR)	The relative (not absolute) difference in events between two treatment groups, expressed as a proportion of the event rate in the untreated group. Similar to RRI, an RRR is therefore a proportional decrease in rates of events between the control and experimental group.
Sensitivity analysis	Process through which the robustness of an economic model is assessed by examining the changes in the result of the analysis when key variables are varied over a specified range.
Special Authority criteria	A subsidy or additional subsidy may only be claimed for certain pharmaceuticals if an application, relating to the specific patient, meeting the Special Authority criteria specified in the Schedule has been approved, and the valid Special Authority number is present on the prescription.
Subsidy	The maximum amount paid to a person, entitled to receive payment from the Crown (usually a pharmacy), for the supply of a pharmaceutical to a patient (this may not be the same as the final cost paid by the Crown, depending on the nature of Pharmac's contractual arrangements with the pharmaceutical supplier).
Technology Assessment Report (TAR)	Documentation of the economic analysis (including cost-utility analysis).
Therapeutic Group Manager (TGM)	Pharmac staff member responsible for managing Pharmac's processes for pharmaceutical funding, within an assigned therapeutic group.
TreeAge™	Decision analysis software used for modelling cost-effectiveness.
Utility	Values of the strength of preferences for, or desirability of, a specific level of health status or a specific health outcome.
Value for money	Refers to whether the benefits of a pharmaceutical are considered significant enough to compensate for the cost.