

PHARMAC  
TE PĀTAKA WHAIORANGA

# Review of the Exceptional Circumstances Framework

**Discussion Document**





## Executive Summary

Pharmac is taking this opportunity to update and improve the **Exceptional Circumstances Framework**<sup>1</sup> (the Framework), which includes the Named Patient Pharmaceutical Assessment (NPPA) policy and other ways Pharmac can fund medicines for individuals in special or exceptional clinical situations.

Under the Pae Ora (Healthy Futures) Act 2022, Pharmac is required to have a system, or framework, that allows people funded access to medicines not listed on the Pharmaceutical Schedule (or listed on the Schedule for a different indication) when their situation doesn't fit our usual funding process.

During the review of the Framework, there will be no changes to how Pharmac assesses applications under the current Framework. Any changes to the Framework will only take place after the review is complete and will be introduced in planned stages to ensure a smooth transition.

Everyone with an existing named patient approval will stay under their current funding arrangements regardless of the outcome of the review.

### What is the Framework?

The Exceptional Circumstances Framework explains how people may be able to access funded medicines when the standard **Pharmaceutical Schedule funding process**<sup>2</sup> does not meet their needs. It is intended to support people with unusual or complex clinical circumstances by setting out the pathways that may be available to them.

The Framework recognises that different funding pathways serve different purposes. The Schedule process supports access for population groups, while other pathways exist to respond to exceptional situations. Together, these processes are designed to work alongside each other so that people can achieve the best possible health outcomes.

The Framework includes the NPPA Policy for people with exceptional clinical circumstances, Special Authority waivers for people who broadly meet the intent of funded criteria but cannot meet every requirement, and other processes that allow Pharmac to consider unique or exceptional situations. These pathways aim to provide flexibility and clarity for people and their clinicians when standard funding rules do not apply.

### How is it funded?

Funding for medicines approved under the Framework comes from Pharmac's overall budget for community and hospital medicines. There isn't a separate ring-fenced fund for the pathways within the Framework, instead, it draws from the same fixed medicine budget that Pharmac uses to fund medicines listed on the Pharmaceutical Schedule.

1 <https://www.pharmac.govt.nz/medicine-funding-and-supply/the-funding-process/policies-manuals-and-processes/exceptional-circumstances-framework-including-the-named-patient-pharmaceutical-assessment-policy>

2 <https://www.pharmac.govt.nz/pharmaceutical-schedule>

## What's included in the review?

The review will look at how the current Framework, including all the funding pathways, is working in practice and where improvements are needed. It will look at what is working well and what could be changed, removed or added, to make things better.

As well as feedback received during consultation, we will also consider the feedback people have already given us to make sure the review reflects what matters most to people.

## Why is Pharmac doing the review?

Pharmac is reviewing the Exceptional Circumstances Framework to ensure it is simpler, clearer, and better meets the needs of people who rely on it to get funded access to medicines. The review is needed to give clinicians a process that is straightforward and easy to navigate, and to give Pharmac clearer criteria and documentation for making timely, consistent decisions while fully meeting legislative requirements.

## What is Pharmac seeking to understand?

This discussion document is part of a broader review of the Framework. Pharmac wants your feedback to help improve the Framework. We're especially interested in hearing:

- how the Framework works for you in practice
- if or how it fits with Pharmac's statutory objectives and priorities
- if it ensures fair access to medicines for people whose clinical needs cannot be met through the Pharmaceutical Schedule
- any gaps, barriers, or unintended consequences you have observed
- suggestions for improving clarity, transparency and consistency in decision making.

## How Pharmac will engage

The review will happen in two stages. In the first stage, Pharmac will gather information on what could be improved. In the second stage, Pharmac will consult again on any proposed changes or improvements informed by the feedback received.

To make sure we are thorough and inclusive, Pharmac will use a range of methods to gather feedback and insights, (see Table One). Consideration will also be given to accessibility needs, for example, different languages, plain English and alternate formats including audio, Easy Read, large print, and New Zealand Sign Language.

**Table One: Planned engagement opportunities**

How you can provide feedback	Description
Webinars <sup>3</sup>	Join live online sessions to learn more and ask questions.
Online feedback forms	Fill out the online survey questions to share your views. The discussion document will be available to view including links to other information, videos and reading materials.
Email	An invitation will be sent out with an opportunity to send us your feedback by email.
Workshops	Take part in hands-on sessions for shared problem-solving.
Visits to identified stakeholders	Pharmac will attend meetings offsite, with times and venues to suit the stakeholder groups.
Direct online conversations and focus groups	One-on-one or small group discussions for detailed insights.
Virtual town hall	Join an open online meeting where you can share your views and hear from others.
Social media	Respond to quick polls or questions on Pharmac's social media channels.

### How decisions will be made

Pharmac wants to be open about how decisions will be made as part of this review. All feedback Pharmac receives during the consultation will be carefully read and considered. We will look for common themes, different views, and any issues that people raise about what is working well and what could be improved.

After the first stage of consultation a summary of feedback will be shared with Pharmac's Senior Leadership Team (SLT). We may also seek advice from specialist groups or people with relevant expertise where this would help us better understand specific issues and feedback received. Following the second round of consultation, there will be further decisions made at SLT. The Pharmac Board will approve the final decisions about any changes and improvements to the policy. Once the review is complete, any changes will be implemented following a clear plan to ensure a smooth transition and keep everyone informed.

<sup>3</sup> What is the difference between a webinar and a virtual town hall? A webinar is a live online session where information is shared about the review. It usually includes a presentation and time for questions at the end. A virtual town hall is a more open online meeting focused on discussion. You can share your views, ask questions, and hear what others think in real time.

# Introduction

## Overview of Pharmac's role

Pharmac was set up in 1993 to provide nationally consistent access to medicines while limiting the rapid growth in expenditure occurring at the time. Pharmac's objective is to secure, for eligible people in need of pharmaceuticals, the best health outcomes that are reasonably achievable from pharmaceutical treatment. It must do this within the fixed budget set by government (the medicines budget).

To achieve its objective, Pharmac manages the Pharmaceutical Schedule (the Schedule) of publicly funded medicines that applies consistently within New Zealand. In addition, Pharmac is responsible for managing national contracts for some medical devices used in public hospitals, ensuring equitable access and cost effectiveness across the health system.

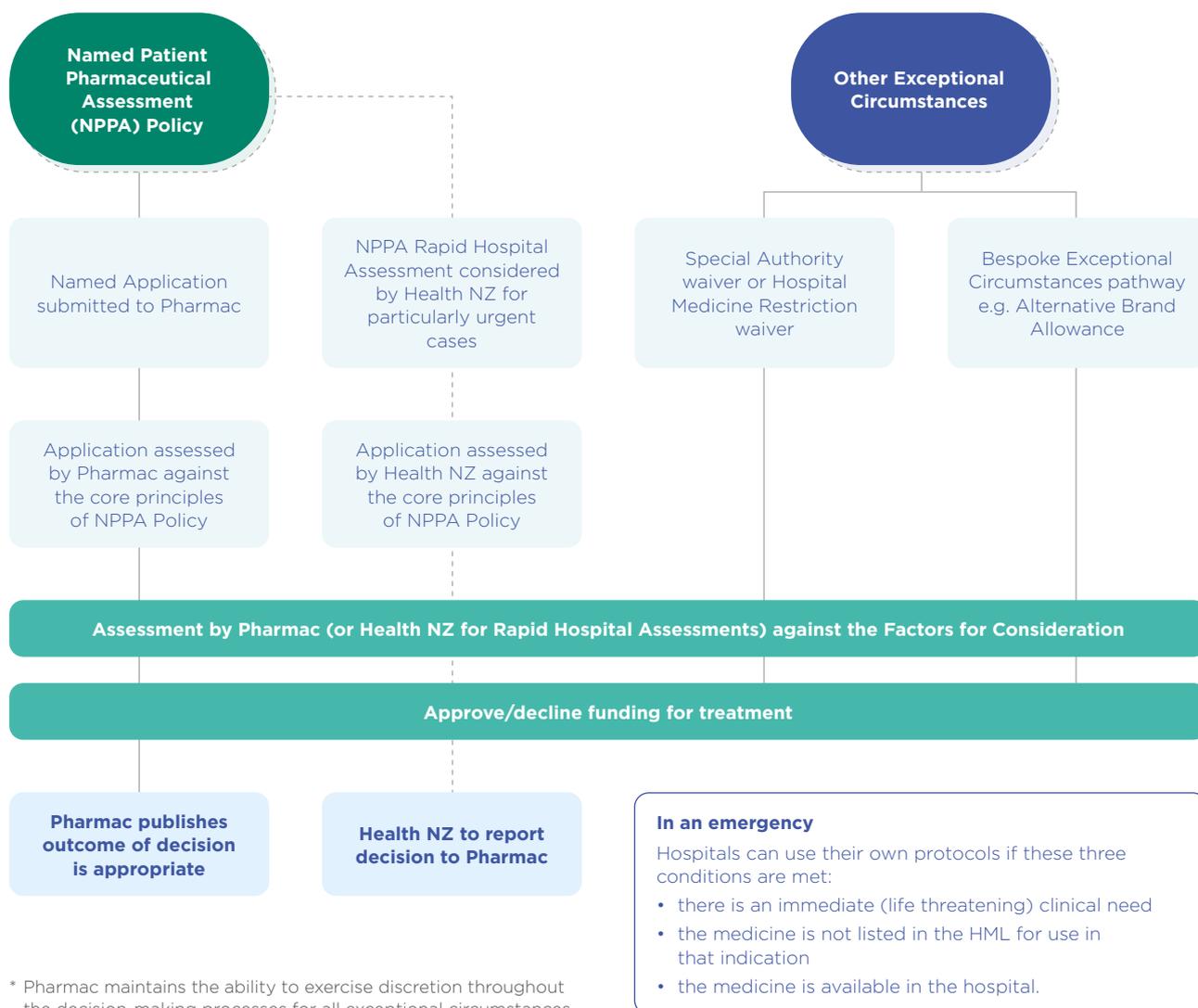
The Factors for Consideration (FFC) (see Appendix One for more detail) are Pharmac's primary decision-making framework and apply to all decisions, including exceptions. The FFC helps Pharmac assess each funding application against **Pharmac's statutory objective**.<sup>4</sup>

## Overview of the Exceptional Circumstances Framework

The Exceptional Circumstances Framework (see Figure One) explains the options available when someone needs medicine funding that does not fit the usual Schedule process. It is designed to help ensure people with exceptional health needs can still access medicines in certain situations. The Framework brings together several pathways, including the NPPA Policy, different types of waivers, requests for alternative brands, and processes that may allow funding for some unapproved medicines when appropriate. These pathways aim to provide clear, flexible options so people can get timely access to the medicines they need.

4 Section 60 of the Pae Ora (Healthy Futures) Act, 2022 states that: The objectives of Pharmac are—(a) 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; and (b) any other objectives it is given by or under any enactment or authorised to perform by the Minister by written notice to the board of Pharmac after consultation with it. In this section, eligible people mean people belonging to a class specified in regulations made under section 102 as being eligible to receive services funded under this Act.

**Figure 1: The current Exceptional Circumstances Framework**



The need to revise the Framework and NPPA policy was identified in the government response to the review of Pharmac in 2022, which directed Pharmac to ‘give high priority to considering equitable outcomes in its decision-making processes and to further develop its analytical frameworks and tools ...to support equitable outcomes’.<sup>5</sup>

## Scope of the review

Pharmac is reviewing the Framework as a whole. Every component of the Framework will be considered, including its principles, criteria, processes, and implementation in practice. The review is not limited to specific issues and is intended to ensure the Framework is effective, equitable, and aligned with Pharmac’s strategic direction and legislative responsibilities.

Medical devices are not within the scope of this current review. However, we welcome comments or feedback, which may be used to inform consideration of a potential future exceptions pathway or process for medical devices.

<sup>5</sup> <https://www.pharmac.govt.nz/about/reset-programme/pharmacreview>

## The aim of the Framework review

Overall, the aim of the Framework review is to:

- review the current NPPA policy and other pathways within the Framework
- determine if the existing Framework adequately meets its legislative function
- improve people's knowledge and understanding of the Framework
- strengthen the Framework and its application
- improve how rare disorders are considered and addressed within the Framework
- increase transparency of the NPPA process to reduce the percentage of applications received that do not meet the principles
- establish policies to reflect processes that have been established due to need (the use of the Framework to manage applications for alternative brands is a good example of this).

## What we've heard so far

Pharmac has already received informal feedback from a wide range of stakeholders including consumers, clinicians, NPPA advisory panel members, Pharmac staff, and others impacted by the Framework. This feedback will be carefully considered, alongside the feedback that will be gathered through this consultation. We will also directly engage with clinicians who don't currently use the process, including those working in primary and rural care and clinicians in the regions where applications are fewer.

Together these insights will help ensure the Framework is effective, equitable, and aligned with Pharmac's strategic direction and legislative responsibilities.

Key themes from feedback received to date include:

- the Framework is not fit for purpose and often misunderstood
- uncertainty about how applications are assessed and when NPPA funding is impacted by Schedule applications
- lack of clarity around what constitutes "exceptional" and how the policy is interpreted to determine this
- confusion about decision-making processes and a lack of transparency
- equity barriers, particularly affecting people with high health needs and other priority populations
- lack of clear guidance and feedback on the application process and decisions made
- inconsistent processes for approving alternative brand allowances.

## Rare disorders

Rare Disorders are defined<sup>6,7</sup> as those affecting one person in every 2,000 or fewer. Ultra-rare disorders are even less common, occurring in one person in every 50,000 or fewer. Funding for medicines for people with rare disorders is considered through the Schedule or through NPPA applications. The main difference is that the Exceptional Circumstances Framework applies to individual people, while rare disorders relate to small groups of people with the same condition. This helps make sure there are clear ways for both individuals and small patient groups to access the treatments they need.

However, even when a condition is classified as rare, it can still affect a relatively large number of people. In some cases, this means the condition may not meet the criteria for NPPA. When this happens, Pharmac considers the group, rather than as individual cases. Although the condition is rare, the total number of patients may be large enough for Pharmac to gather evidence and expert advice through the standard Pharmaceutical Schedule process. At present, there is no specific pathway under the Exceptional Circumstances Framework for funding medicines for rare disorders.

6 <https://www.health.govt.nz/publications/aotearoa-new-zealand-rare-disorders-strategy>

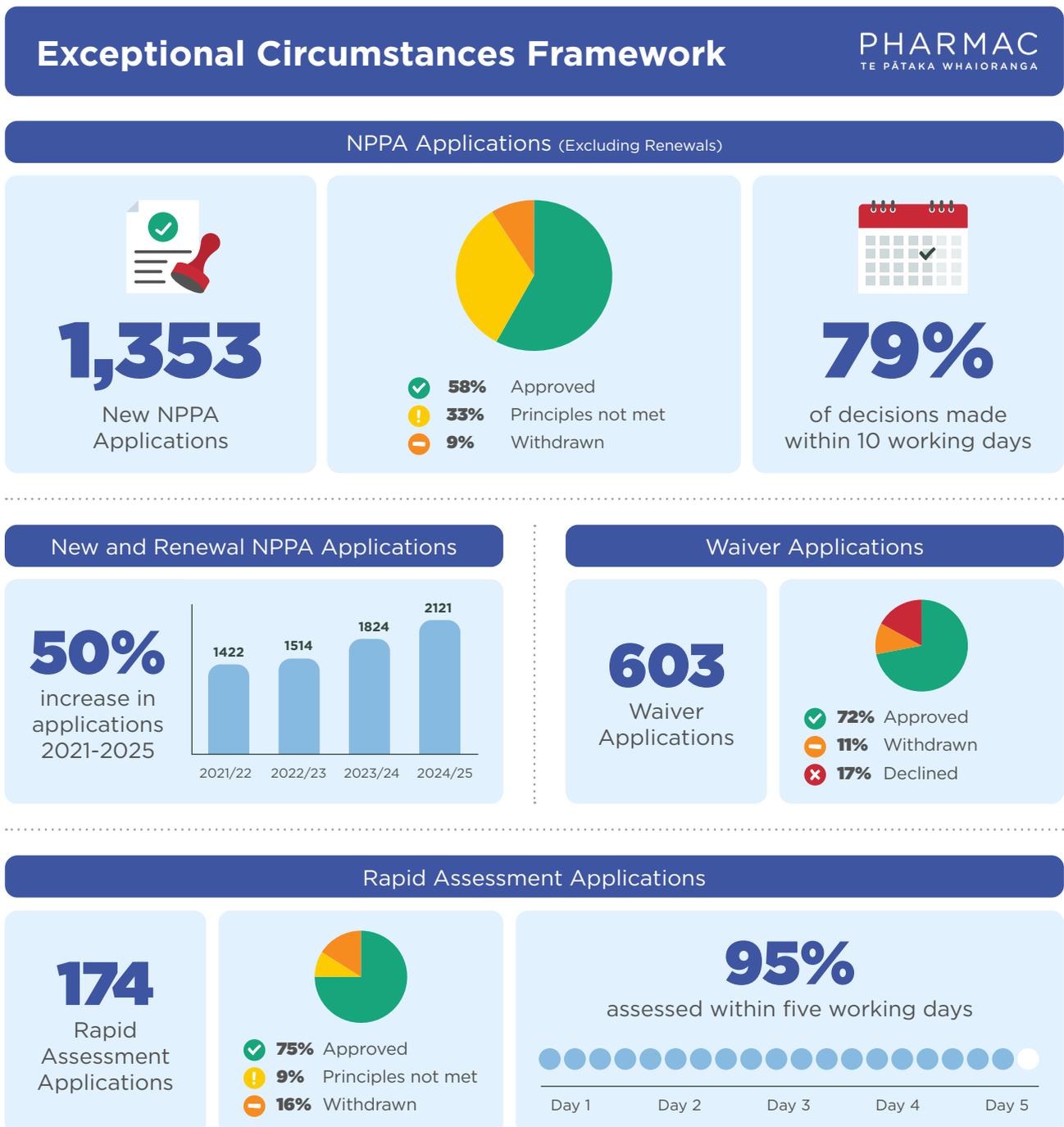
7 <https://www.pharmac.govt.nz/medicine-funding-and-supply/the-funding-process/from-application-to-funded-medicine-how-we-fund-a-medicine/medicines-for-rare-disorders>

# Exceptional Circumstances Framework – pathways

There are several pathways within the Exceptional Circumstances Framework. These pathways allow people funded access to medicines not listed on the Pharmaceutical Schedule (or listed on the Schedule for a different indication) when their situation doesn't fit our usual funding process.

The infographic (Figure 2) summarises key information about the use of the Exceptional Circumstances pathways, offering a clear snapshot of the volume and nature of applications.

**Figure 2: Exceptional Circumstances Framework - Snapshot (2024/2025)**



# Named Patient Pharmaceutical Assessment (NPPA)

## For people with exceptional clinical circumstances

Pharmac developed the NPPA policy to guide how it makes decisions about funding medicines for people in exceptional circumstances. It is designed for people who have urgent and unusual clinical needs that are not met by treatments currently funded through the Schedule. Before applying for NPPA, all suitable funded treatments must have been tried. If there are other funded options available, they need to have been used and/or be clinically unsuitable for the person's specific situation.

## How NPPA works

Pharmac (or Health New Zealand hospitals, in urgent hospital cases) uses three core NPPA Principles (see Appendix Two) to decide whether a treatment should be considered for funding through the NPPA process. If an application meets these Principles, it will then be assessed using Pharmac's FFC.

Pharmac can also decide that a different funding process might be more suitable for a particular application. Even if an application does not meet the NPPA policy principles, Pharmac still has the option to fund a treatment using wider discretion if the situation is appropriate.

## How Pharmac processes a NPPA application

Depending on the complexity of the application, the assessment process can involve input from several Pharmac staff and external clinical experts.

Exceptions advisors assess NPPA applications and may seek input from Pharmac Therapeutic Group Managers (TGMs). Clinical advice on individual applications may also be sought from the NPPA Advisory Panel, members of the Pharmacology and Therapeutics Advisory Committee (PTAC), a Specialist Advisory Committee, or other independent experts.

## When NPPA is not the right pathway

This policy is not intended for treatments that have already been considered for funding through the Schedule for the same condition. It also does not apply where there is already a suitable funded treatment available or when the treatment would be suitable for many people. However, if a person's clinical circumstances differ from those previously assessed, for example their health need is significantly different or they cannot use the currently funded treatment, this may progress for assessment under the FFC.

## NPPA sets a standard

When an application is approved under this policy, it sets a precedent. This means that future applications for the same treatment, for people in similar circumstances, are also likely to be approved. This includes when a Schedule application has been received after approval under NPPA.

## NPPA Rapid Assessment applications

Rapid assessments are applications for treatments used within Health New Zealand hospitals in situations that are considered clinically urgent. Pharmac commits to making decisions on such applications in less than five working days (noting that if a hospital requires a decision faster they are authorised to make the decision locally).

## Financial implications for NPPA

Funding for NPPA-approved applications comes from the medicines budget with no separate or ring-fenced allocation. This means that treatments funded by both NPPA and the Schedule come from the same budget. NPPA-related expenditure has grown over time, driven by an increase in application volumes and a rise in high-cost medicines being assessed through the Framework.

## Other Exceptional Circumstances

### Special Authority waiver and Hospital Medicine Restriction waivers

If a person's circumstances align with the intent of a Special Authority or Hospital Medicine Restriction waiver, but do not fully meet the stated requirements, prescribers may request that those requirements be waived.

Sometimes the usual requirements cannot be met safely or appropriately. For instance, some medicines require another treatment to be tried first. Methotrexate is one example of this, as it cannot be used during pregnancy, a waiver may be appropriate for people who are pregnant or planning pregnancy. These decisions typically have no fiscal impact, as funding has already been approved; the focus is on whether the person's situation meets the intent of the group eligible for funding.

### Bespoke exceptional circumstances pathway

Sometimes there is a need for Pharmac to change the funded brand of medicine. When this happens Pharmac understands the change may not be suitable for everyone. Currently, one way to fund alternative brands, is through a bespoke framework pathway. This allows people to remain on their current treatment when it's not clinically suitable for them to transition to the newly funded brand.

The pathways for some applications, such as alternative brand requests for diabetes technology, are bespoke and have their own application forms and pharmaceutical-specific assessment criteria.

### Automatic approvals

The Framework also allows some medicines that are not approved by Medsafe and are not listed on the Schedule to be funded, without needing a full assessment. This is called an automatic approval. This pathway is used when a medicine is well-established, at low risk, and commonly needed, so requiring a full assessment would add unnecessary delays. An example is the routine approval of oral vitamin A and E.

# Review Approach

Pharmac is reviewing the NPPA policy and the broader Framework following longstanding calls for change. We are now able to undertake this review and are seeking feedback from stakeholders.

The aim of this consultation is to provide clear, accessible opportunities for everyone to share their views. While feedback can be given through our website and email, we know these channels may not reach all key stakeholders. To ensure broad engagement, we will also hold targeted sessions, hui, and work through trusted networks, offering flexible ways for people to participate.

The consultation period will be open for a set timeframe, although timelines (in Table Two) may be subject to change depending on the nature and volume of feedback received.

The first step is the release of this Discussion Document and an online survey. Other methods will also be used to gather feedback, for example, webinars and workshops. This stage focuses on sharing information and gathering feedback from all stakeholders. Pharmac will use this input to identify improvements and develop options. A second consultation will follow on proposed changes.

This two-stage public consultation has the expected benefits of:

- providing Pharmac with additional information that enhances analysis and decision-making process
- allows for a more open, consultative process where stakeholders can be more involved throughout the process on a sensitive subject matter
- stakeholders can feed in earlier in the policy process, reducing risks that Pharmac has pre-determined an outcome.

This approach will allow for more meaningful feedback, which should enable Pharmac to develop a solution that is robust, equitable and sustainable. Pharmac is open to seeking feedback on any aspect of the Framework from all stakeholders.

**Table Two: Framework review timeline**

Stages		
Public consultation on discussion document	Key Steps	<ul style="list-style-type: none"> <li>• Start consultation</li> <li>• Discussion document on website</li> <li>• Feedback methods established and set up</li> </ul>
	Estimated Time	<b>Consultation will be open for 12 weeks.</b>
Submissions	Key Steps	<ul style="list-style-type: none"> <li>• Summarise submissions received. Follow up with stakeholders on their submissions as necessary (for example, clarifications).</li> <li>• Post information gathered on submissions received on Pharmac’s website.</li> </ul>
	Estimated Time	<b>8 weeks</b>
Drafting proposed changes paper	Key Steps	<ul style="list-style-type: none"> <li>• Draft paper, which includes Pharmac preferred option/s and implementation considerations.</li> <li>• Internal consultation on draft paper including approval from SLT.</li> </ul>
	Estimated Time	<b>4 weeks</b>
Public consultation on proposed changes paper	Key Steps	<ul style="list-style-type: none"> <li>• Meet with key stakeholders and submitters</li> <li>• Release of a proposal paper</li> <li>• Consultation and feedback</li> </ul>
	Estimated Time	<b>8 weeks</b>
Submissions analysis and draft documents final decision	Key Steps	<ul style="list-style-type: none"> <li>• Summarise submissions received. Follow up with stakeholders on their submissions as necessary (for example, clarifications).</li> </ul>
	Estimated Time	<b>10 weeks (note: Pharmac closed for 2 weeks over Christmas/ New Year)</b>
Final decision	Key Steps	<ul style="list-style-type: none"> <li>• Decision paper for SLT and Board</li> <li>• Aim for Board Paper in March</li> <li>• Announcement of decision by Pharmac.</li> </ul>
	Estimated Time	<b>10 weeks</b>

# Feedback received to date

Following feedback received to date, general considerations were developed for the review include the following topics.

## 1. Consider whether the current Framework and the principles are fit for purpose

Currently, about a third of NPPA applications (excluding renewals) are found not to meet the three Principles used to determine whether to progress an application before they are viewed against the FFC, and a decision made by the Board or its delegate is required.

As noted, the exceptional circumstances team consulted with clinicians to understand the limitations of the application process and one of the key areas that was highlighted for improvement was to improve clinicians' understanding of the NPPA policy. When looking at the Principles in isolation, clinicians can feel unclear about what is required or how Pharmac interpret the policy to determine what is exceptional.

The pathways for some applications, such as alternative brand requests, are bespoke and have their own application forms and pharmaceutical-specific assessment criteria. Mechanisms to manage requests for alternative brands are set up on an ad-hoc basis where it is identified that a brand change may significantly impact a patient group. The creation of a bespoke pathway is often resource intensive, with no predetermined mechanism for these pathways. It can also be difficult to assess applications under a different set of criteria each time one is created.

Applications for cancer treatment are becoming more specialised and complex and can be challenging to apply the NPPA policy too, as the range of genetic tests and targeted treatments increases. Treatments like immune checkpoint inhibitors that work well for many different types of cancer, add to the complexity.

Applications for rare disorders can also be challenging under the current NPPA policy because these conditions often affect small patient groups, resulting in limited clinical evidence to support funding decisions. Schedule applications often remain on the Options for Investment (OFI) for some time due to the high cost and limited evidence for treatment. This creates a gap where NPPA funding may be restricted, yet no Schedule application is actively considered, leaving patients with few viable pathways for access.

## 2. Consider societal impact

The Government's 2025/26 **Letter of Expectations**<sup>8</sup> suggested Pharmac include the wider fiscal impacts to Government, and broader societal and non-health outcomes, of funding medicines and medical devices. This includes assessment and decision-making to account for fiscal benefits beyond the health sector and to consider the broader economic and societal benefits of medical technologies.

Rather than health technology assessments considering only health system perspectives and impacts, a societal approach includes all costs and benefits associated with a policy intervention. This perspective considers not only direct medical costs and health outcomes but also indirect costs and benefits such as productivity losses or gains, social care costs, and broader economic impacts on various sectors of society.

As part of this review, any changes to the Framework must align with the Government's request for Pharmac to update its decision-making and evaluation models to consider the broader fiscal and societal impacts of funding decisions.

<sup>8</sup> <https://www.pharmac.govt.nz/news-and-resources/publications/corporate-publications/letter-of-expectations>

### 3. Consider inequities in the current Framework and ensure accessibility in applications to those with high health needs

Currently the NPPA policy requires applications to be submitted on a patients' behalf by any clinician, within their scope of practice. Currently approximately 75% of applications are submitted by a secondary or tertiary care specialist (both in public and private settings). This can create inequities depending on a patient's ability to access these services. The review will provide an opportunity to explicitly incorporate principles and/or assessment processes to ensure that those with the greatest health need are considered.

The NPPA application and assessment process doesn't ask for patient ethnicity information; however, we are able to find this information from NHI data which can be gathered for reporting purposes. In 2022/23, a report was requested regarding the number of applications for those who identify as Māori. It was noted that a greater proportion of NPPA applications for people who identify as Māori were approved (75%) compared to the average (63%). However, it appears that there may be slightly fewer NPPA applications submitted for Māori compared to the general population. In the last financial year, 16% of NPPA and waiver applications were submitted for Māori while 17% of the population identify as Māori.<sup>9</sup>

Currently Pharmac do not have data on any other priority populations but intend to include this work as part of the revision of the Framework.

### 4. Consider the use of discretion as part of assessing applications

The review will examine where and how discretion is currently applied in the assessment of applications, and the guidelines under which this occurs. While discretion is used in specific and limited circumstances, this is not always clearly articulated in existing guidance. As part of the review, we aim to develop clearer and more explicit guidance for consumers, applicants and Pharmac staff to support greater transparency and consistency in decision-making. This will include clarifying the circumstances in which discretion may be applied, how it is assessed, and how this is communicated to applicants, with a view to improving understanding of the process and increasing confidence in the application of the Framework.

<sup>9</sup> <https://www.stats.govt.nz/information-releases/maori-population-estimates-at-30-june-2024/>

# Questions for Consultation

Pharmac is asking for input to help better understand the complex issues related to the Framework and its processes, including the NPPA Policy. This section outlines key issues and questions for feedback.

## Exceptional Circumstances Framework

Pharmac's role includes considering whether to fund pharmaceutical treatments for people with **exceptional circumstances** when those treatments are not currently available for them on the Pharmaceutical Schedule.

This role reflects the legislative function outlined in section 69(1)(b) of the Pae Ora (Healthy Futures) Act 2022:

*[Managing] incidental matters arising out of [maintaining and managing a pharmaceutical schedule], including in exceptional circumstances providing for subsidies for the supply of pharmaceuticals not on the pharmaceutical schedule.*

### General Exceptional Circumstances Framework Questions:

What is your experience of the Framework?

Comment:

What do we need to change?

Comment:

What improvements would you consider?

Comment:

Do you feel the current system supports equitable access to medicines for all New Zealanders?

Yes/No

Comment:

Is the current information on Pharmac's website easy to understand and navigate?

Yes/No

Comment:

## Named Patient Pharmaceutical Assessment

Pharmac uses its Named Patient Pharmaceutical Assessment (NPPA) policy to consider whether to fund a treatment for an individual patient whose clinical circumstances are exceptional.

The NPPA policy has three principles.

1. The NPPA policy provides a pathway to consider those whose clinical circumstances cannot be met through the Pharmaceutical Schedule at a given point in time
2. The NPPA policy complements the Pharmaceutical Schedule and the Schedule decision-making process
3. The NPPA policy is designed for individual assessment.

All three principles must be met for an application to be progressed for a decision.

What is your experience of NPPA?

Comment:

Do you consider the three principles to be fit for purpose?

Yes/No

Comment:

If you consider one or more of the principles to be not fit for purpose, what would you like to see changed?

Comment:

How confident are you in interpreting the NPPA principles?

Not at all confident

Slightly confident

Moderately confident

Very confident

Extremely confident

### NPPA application policy questions to interpret the principles.

The following questions are used by Pharmac staff to help interpret the principles of the NPPA Policy:

1. Does the person have exceptional clinical circumstances?
2. Has the person tried all existing funded alternative treatments?
3. Has Pharmac considered the treatment for funding previously?

In your view, do these questions provide clear and useful guidance for interpreting each of the three NPPA principles in practice?

Comment:

Can you provide a specific example of a situation where the NPPA policy has been difficult to interpret?  
(withhold any identifiable information)

Comment:

## Application and decision

After Pharmac receives the application, we assess whether it meets the principles of the NPPA Policy.

If the principles are not met, Pharmac will contact the clinician who made the application. Pharmac may invite them to provide more information. If the principles are met, Pharmac will then consider the application against the FFC (see Appendix one). Pharmac may also seek clinical advice from the NPPA Advisory Panel to help apply the Factors.

Do you have any comments on the application process itself?

Comment:

Are you confident accessing the correct forms and completing the submission?

Not at all confident

Slightly confident

Moderately confident

Very confident

Extremely confident

Were you kept informed about the progress and outcome of the application?

Yes/No

Comment:

Is the current information available on the Pharmac website regarding NPPA easy to understand and navigate?

Yes/No

Comment:

## NPPA Rapid Assessment Pathways

In clinically urgent situations, Health NZ hospitals can access medicines through one of three pathways: **NPPA rapid assessment** by Pharmac, **in-hospital rapid assessment** by Health New Zealand, or the **emergency-use rule** for immediate treatment access, by Health New Zealand.

Clinicians use a NPPA rapid assessment application when:

- treatment is to be administered to an inpatient of a Health NZ hospital
- the situation is clinically urgent.

As a guide, if the treatment needs to start within 5 working days because of clinical urgency, then NPPA rapid assessment by Pharmac may be appropriate. Where Health NZ hospitals have a specific rapid assessment process, an in-house rapid assessment can be completed. The application still needs to be assessed using the principles of the NPPA policy. Health NZ hospitals report the outcomes of these assessments to Pharmac.

Pharmac (rather than the hospital) must assess any rapid assessment applications for:

- any pharmaceutical cancer treatment
- rituximab, infliximab, tocilizumab, bevacizumab.

## Emergency use rule

Health NZ use their usual hospital protocols (rather than a Rapid pathway) if these three conditions are met:

- there is an immediate (life threatening) clinical need
- the medicine is not listed on the Schedule for use in that indication
- the medicine is available in the hospital.

The Framework also provides a mechanism for Pharmac to fund treatments for specific patients outside of the Pharmaceutical Schedule and separate to the NPPA Policy. These pathways are currently classified as 'others' in the Framework and include Special Authority Waivers and a few bespoke pathways created in response to a brand change.

What is your experience with the Rapid Assessment pathways?

Comment:

When do you consider using hospital rapid assessments vs the emergency use rule and why?

Comment:

Is it clear which pathway to use in an urgent situation?

Yes/no

I have a clear understanding of the NPPA rapid assessment process

No understanding                      Minor understanding                      Moderate understanding                      Full understanding

I have a clear understanding of the in-hospital rapid assessment process

No understanding                      Minor understanding                      Moderate understanding                      Full understanding

I have a clear understanding of the current emergency use rule

No understanding                      Minor understanding                      Moderate understanding                      Full understanding

## Bespoke exceptional circumstances

Please share your experience with the bespoke exceptional circumstance's pathway, including any challenges or areas of clarity.

I have a clear understanding of the bespoke exceptional circumstances assessment process (e.g. alternative brand application)

No understanding                      Minor understanding                      Moderate understanding                      Full understanding

The information available on Pharmac's website regarding bespoke exceptional circumstances is easy to understand and navigate.

Strongly disagree                      Disagree                      Neutral                      Agree                      Strongly agree

Bespoke exceptional circumstances pathways have been created to support brand changes. These pathways in general have their own application form, and criteria for assessment that are specific to the pharmaceutical. There are no set criteria across these pathways for assessment of alternative brand requests.

## Special Authority waivers and Hospital Medicine Restriction waivers

Some pharmaceuticals that are listed in the Schedule require certain conditions (Special Authority criteria) to be met before funding is granted. These conditions generally ensure that funding is targeted to those patients that would benefit most from treatment.

If a patient's clinical circumstances align with the intent but not the exact criteria of Special Authority waiver or Hospital Medicine Restriction waiver, prescribers can apply.

Please share your experience with the Special Authority waivers or Hospital Medicine Restriction Waivers including any challenges or areas of clarity.

Comment:

I have a clear understanding of the waivers

No understanding

Minor understanding

Moderate understanding

Full understanding

The information available on Pharmac's website regarding the waivers is easy to understand and navigate.

Strongly disagree

Disagree

Neutral

Agree

Strongly agree

## Additional questions

Do you see the need for additional exceptional circumstances framework pathways? If so, what?

Comment:

Do you consider that different clinical groups would need their own exceptional circumstances pathway?

Comment:

Are there any current pathways in the Framework you would like to remove or see changes to? If so, provide more information.

Comment:

What improvements would you suggest for the application form or IT systems?

Would more detailed reporting (e.g. reasons for 'principles not met' (PNM) decisions) help you decide whether to apply?

Comment:

## Do you have other comments?

# Your feedback may be shared

When you give feedback on a consultation, your feedback becomes official information that Pharmac holds. Pharmac has legal responsibilities for how we manage this official information, under laws such as the Official Information Act and Privacy Act.

Pharmac may receive a request from people for official information, which could include your feedback. Legally, Pharmac must consider whether your feedback should be released.

We will consider your views when assessing whether the feedback has to be released. Tell us if there is anything about your feedback that you would prefer wasn't released.

If your feedback is proposed for release, then Pharmac will contact you, unless there is a legal reason that we can't.

Note that Pharmac collects and holds your information in line with our [\*\*Privacy Statement\*\*](#).<sup>10</sup>

<sup>10</sup> <https://www.pharmac.govt.nz/about-this-site/privacy-statement>

# Exceptional Circumstances Framework Review

## Full Summary of Questions

### General

What is your experience of the Framework?

What do we need to change?

What improvements would you consider?

Do you feel the current system supports equitable access to medicines for all New Zealanders?

Is the current information on Pharmac's website easy to understand and navigate?

### Named Patient Pharmaceutical Assessment

What is your experience of NPPA?

Do you consider the three principles to be fit for purpose?

If you consider one or more of the principles to be not fit for purpose, what would you like to see changed?

How confident are you in interpreting the NPPA principles?

Not at all confident

Slightly confident

Moderately confident

Very confident

Extremely confident

In your view, do these questions provide clear and useful guidance for interpreting each of the three NPPA principles in practice?

Can you provide a specific example of a situation where the NPPA policy has been difficult to interpret?  
(withhold any identifiable information)

Do you have any comments on the application process itself?

Are you confident accessing the correct forms and completing the submission?

Not at all confident

Slightly confident

Moderately confident

Very confident

Extremely confident

In general, were you kept informed about the progress and outcome of the application?

Is the current information available on Pharmac's website regarding NPPA easy to understand and navigate?

## Named Patient Pharmaceutical Assessment

What is your experience with the Rapid Assessment pathways?

When do you consider using hospital rapid assessments vs the emergency use rule and why?

Is it clear which pathway to use in an urgent situation?

I have a clear understanding of the NPPA rapid assessment process

No understanding

Minor understanding

Moderate understanding

Full understanding

I have a clear understanding of the in-hospital rapid assessment process

No understanding

Minor understanding

Moderate understanding

Full understanding

I have a clear understanding of the current emergency use rule

No understanding

Minor understanding

Moderate understanding

Full understanding

## Bespoke exceptional circumstances

Please share your experience with the bespoke exceptional circumstance's pathway, including any challenges or areas of clarity.

I have a clear understanding of the bespoke exceptional circumstances assessment process (e.g. alternative brand application)

No understanding

Minor understanding

Moderate understanding

Full understanding

The information available on Pharmac's website regarding bespoke exceptional circumstances is easy to understand and navigate.

Strongly disagree

Disagree

Neutral

Agree

Strongly Agree

## Special authority waivers and Hospital Medicine Restriction waivers

Please share your experience with the Special Authority waivers or Hospital Medicine Restriction Waivers including any challenges or areas of clarity.

Comment:

I have a clear understanding of the waivers

No understanding

Minor understanding

Moderate understanding

Full understanding

The information available on Pharmac's website regarding the waivers is easy to understand and navigate.

Strongly disagree

Disagree

Neutral

Agree

Strongly Agree

## Special Authority waivers and Hospital Medicine Restriction waivers

Please share your experience with the Special Authority waivers or Hospital Medicine

### Restriction Waivers including any challenges or areas of clarity.

I have a clear understanding of the waivers

No understanding

Minor understanding

Moderate understanding

Full understanding

The information available on Pharmac's website regarding the waivers is easy to understand and navigate.

Strongly disagree

Disagree

Neutral

Agree

Strongly Agree

### Questions

Do you see the need for additional exceptional circumstances framework pathways? If so, what?

Do you consider that different clinical groups need their own exceptional circumstances pathway?

Are there any current pathways in the Framework you would like to remove or see changes too?  
If so, provide more information.

What improvements would you suggest for the application form or IT systems?

Would more detailed reporting (e.g. reasons for 'principles not met' (PNM) decisions) help you decide whether to apply?

# Appendix One

## Factors for Consideration



**Need** - to work out what the level of 'need' is we consider the impact of the disease, condition or illness on the person, their family or whānau, wider society, and the broader New Zealand health system.

**Health Benefits** - this is about the potential health gain from the medicine being considered.

**Costs and savings** - we consider the costs and savings to the person and their family, whānau and to wider society. The cost and savings to the health system covers both the pharmaceutical budget and the wider health system.

**Suitability** - this considers the non-clinical features of the medicine or medical device that might impact on health outcomes.

# Appendix Two

## Core principles of the NPPA Policy

### **1. The NPPA Policy provides a pathway to consider those whose clinical circumstances cannot be met through the Pharmaceutical Schedule at a given point in time**

Pharmac recognises that there are some clinical circumstances for which a given treatment is not available through the Pharmaceutical Schedule or cannot feasibly be considered through the Pharmaceutical Schedule listing process at a given point in time. The NPPA Policy process in combination with the Pharmaceutical Schedule listing process ensures there is a 'door' to consider funding for all patients.

### **2. The NPPA Policy complements the Pharmaceutical Schedule and the Schedule decision-making process**

The NPPA Policy must operate only to consider those funding applications which are not appropriate to be considered through the Pharmaceutical Schedule listing process, for example due to the highly unusual nature of an individual's clinical circumstances. This ensures that the NPPA Policy does not undermine the Schedule through providing an alternative competing pathway for funding in the same circumstances.

### **3. The NPPA Policy is designed for individual assessment**

While the Pharmaceutical Schedule lists medicines for population groups, the NPPA Policy considers applications to fund treatments for individuals. This acknowledges that some individuals have specific clinical circumstances that may be different to a wider patient population. However, if a person has the same clinical circumstances as a wider patient population, the Pharmaceutical Schedule may be the more appropriate pathway to consider these circumstances so that all people with the same condition have equal opportunity to access the treatment.

## Keep in touch

---

**The views of people who may be impacted by the decisions we make are important to us.**

Find out how to get in touch on our website [pharmac.govt.nz/contact](https://www.pharmac.govt.nz/contact)

Email us directly at [ecfreview@pharmac.govt.nz](mailto:ecfreview@pharmac.govt.nz)

**Or send a hard copy of your feedback to:**

Pharmac  
PO Box 10254  
The Terrace  
Wellington 6143

---

**PHARMAC**  
TE PĀTAKA WHAIORANGA

**Te Kāwanatanga o Aotearoa**  
New Zealand Government

