

Frequently Asked Questions about the Exceptional Circumstances Framework and Current Review

This FAQ brings together questions you've asked during our engagement and other common questions about the Exceptional Circumstances Framework (the Framework)¹.

It aims to help address common questions about how the Framework works and about the current review.

We'll keep updating these FAQs on the website: www.pharmac.govt.nz/ECF-FAQ.

¹ The Exceptional Circumstances Framework outlines the ways in which Pharmac generally considers funding decisions for exceptional circumstances that fall outside of the Pharmaceutical Schedule funding process, and guides Pharmac's decision making in these cases.

1. How is an application assessed?

- Each application under the Framework is assessed:
 - on its own merits
 - based on the information available at the time
 - in line with the policy
- This means outcomes for one person do not guarantee the same outcome for others.

2. Who can make an application under the Exceptional Circumstances Framework?

- Applications made under the Framework must come from an authorised prescriber.
- Any authorised prescriber can apply if the request is within their scope of practice.

3. Does Pharmac stop funding a medicine through Named Patient Pharmaceutical Assessment after receiving a Schedule funding application?

- No. Anyone with a current Named Patient Pharmaceutical Assessment (NPPA) approval keeps funded access to the medicine for as long as they meet funding criteria.
- For any new patients, if their clinical circumstances are similar to a previously approved application (and the other principles are met), Pharmac may continue to fund treatment through NPPA.
- On occasion, we start funding a treatment for a small number of people because we have had advice that their circumstances are exceptional. We can then discover that a larger group of people with similar circumstances could benefit, usually because we have received a lot of applications. If this happens, Pharmac will seek new clinical advice.

- If that advice shows that circumstances are no longer exceptional, Pharmac may consider that the medicine is better funded through the Schedule. If this is the case, we will stop funding new patients through NPPA. People with existing NPPA approvals will continue to get funded access to the medicine, for long as they meet the funding criteria.

4. What is the difference between a 'Decline' and 'Principles not met' outcome for a NPPA application?

- While the outcome for the patient is the same (that the medicine is not funded) the reason differs.
- There are two stages to our consideration of a NPPA application:
 - First, are the principles of the NPPA policy met?
 - Then, if yes, the application is assessed against the Factors for Consideration (FFC)².
- If an application does not meet the principles of the NPPA policy, it does not go to the assessment stage. This is reported as "principles not met".
- A "Decline" only occurs if, after assessing an application against the FFC, Pharmac decides not to fund the medicine.
- Declining applications is rare. We have declined fewer than 1% of applications.

5. Does Pharmac apply discretion when assessing NPPA applications, how, and under what guidelines.

- Under the current Framework, Pharmac can apply discretion to fund treatments for people, even where the circumstances do not fully meet the core principles of the NPPA policy.
- We may use discretion when a funded alternative is more expensive than the treatment being sought through a NPPA application and all other principles are met. The person would still need to have exceptional clinical circumstances.

- As part of the review, Pharmac will develop clearer guidance for consumers, prescribers, and Pharmac staff. We want to reduce our reliance on discretion and improve transparency about when and how it is applied.

6. What is the number of people in a patient group that would make a situation exceptional?

- There is no fixed patient number. Guided by clinical advice, Pharmac looks to the principles of the NPPA policy and considers whether a patient group could be funded through the Schedule or if an exceptions pathway is needed. Patient numbers is not the only reason that someone is or isn't clinically exceptional.

7. Does Pharmac consider every application in isolation?

- We consider every NPPA application individually. NPPA decisions are informed by, not determined by, earlier decisions. A single application doesn't provide access for a group of people with similar circumstances.
- Where we have considered NPPA applications with similar clinical circumstances, you can expect similar funding decisions.
- However, evidence can change over time, which may lead to a different decision. For example, where there is new evidence to support a treatment, a different recommendation may be made using the FFC.
- As such, Pharmac considers every application individually, but not in isolation.

8. How does Pharmac get the right clinical advice?

- Pharmac's main source of clinical advice for NPPA applications is the [NPPA Advisory Panel](#). The Panel is made up of 13 clinicians, covering 10 specialities.
- Three members of the Advisory Panel provide advice three times a week, on a rostered basis. All three members give advice on each application individually, and Pharmac considers this as a collective clinical view.
- When more specialist input is needed, we seek advice from members of our Special Advisory Committees. If we still can't find a suitable expert, we work with the applicant to make sure we get appropriate specialist advice. We have strengthened this collaborative approach in response to feedback.
- When communicating the outcome of an application, Pharmac explains where the advice came from and provides a summary. This helps applicants understand the basis of the decision and raise any concerns if needed.

9. Are the Principles of the NPPA policy up for review through this process?

- Yes, the NPPA policy, including the Principles are in scope of the review.

10. If Pharmac has flexibility to consider exceptional circumstances and wider impacts, why is it still hard for medicines for rare disorders to be funded?

- Under the current NPPA policy, we can fund applications for rare disorders where the principles are met and the application has been successfully considered against the FFC.
- As part of this review, Pharmac wants to explore whether we need more pathways to better meet our legislative responsibilities under the Pae Ora

² The Factors for Consideration is the framework Pharmac uses when making funding decisions.

(Healthy Futures) Act.

- This could include specific pathways within the Framework for rare disorders, cancer treatments, or other defined exceptional circumstances.

11. Are equity considerations taken into account when assessing applications under the Exceptional Circumstances Framework?

- We take equity considerations into account through our FFC, which are used in all decisions. The Factors include health need, health benefit, cost and savings, and suitability, at both individual and population levels.
- Under the current Framework, equity is not explicitly addressed at the policy level. As part of any policy review, Pharmac would consider how populations with the greatest health needs could be better reflected at a policy level.

12. Does Pharmac report on, or publish, the outcomes of Exceptional Circumstances applications?

- We publish outcome data for initial NPPA applications for the last four financial years on [our website](#).
- To protect patient privacy and align with Pharmac's privacy policy, we do not report outcomes where there are fewer than seven similar requests.

13. Where does the funding come from for treatments approved under the Exceptional Circumstances Framework?

- The medicines budget funds treatments approved through the Framework. There is no fixed or ring-fenced fund for treatments funded under the Framework.
- The budget and how medicines are funded are out of scope of this review.

14. Why are there renewals each year, are renewals only given for one year?

- Renewal criteria help Pharmac assess whether the intended benefits of treatment are being achieved, similar to [Special Authority criteria](#)³.
- Approval durations vary depending on the treatment and condition. Durations can vary anywhere from 1 month to lifetime. Where a patient is stable and their condition is unlikely to change, we may consider approvals for several years or for the person's lifetime.

³ Pharmac sets specific criteria that must be met before some medicines will be funded. We do this to ensure medicines are targeted to those who would benefit most from treatment.