

Review of Exceptional Circumstances Framework

Submission by Prader-Willi Syndrome Association NZ Inc.

Submitted via email to: ecfreview@pharmac.govt.nz

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Thank you for the opportunity to provide feedback on Pharmac's review of the Exceptional Circumstances Framework.

Prader-Willi Syndrome Association New Zealand (PWSA NZ) represents New Zealanders living with Prader-Willi syndrome (PWS) and their families and whānau. PWS is a rare genetic disorder associated with complex lifelong health needs, which include endocrine, metabolic, behavioural and mental health challenges.

We welcome Pharmac's stated objectives for this review, particularly its commitment to improving clarity and understanding of the framework, strengthening the policy settings, increasing transparency, and improving how rare disorders are considered and addressed within the framework.

We believe the current framework does not adequately address the circumstances faced by people living with rare disorders such as PWS.

While the Named Patient Pharmaceutical Assessment (NPPA) process can provide an important pathway for access, patient and family awareness of the framework is limited, and access is also heavily dependent on clinicians being aware of the process and willing to make an application. This creates inequities, particularly for people who do not have access to relevant specialists. Despite international recommendations that adults with a PWS diagnosis continue to see a specialist for annual monitoring, this often does not occur in New Zealand, and patients may be dependent on GP monitoring alone. Therefore, one patient with PWS may have an application submitted by their specialist whilst another patient with similar individual need who only sees a GP would not. More transparency around potential availability of treatments is needed.

We are also concerned that there remains a lack of clarity around what constitutes an "exceptional" circumstance and how discretion is applied. Greater transparency would help patients, clinicians and advocacy organisations understand when an application may be appropriate and what factors are likely to influence decisions. Pharmac has itself identified confusion around the meaning of "exceptional", and through the review process, transparency of decision-making and equity barriers were also raised as key issues.

As new treatments for rare disorders emerge, it is likely that many patients will continue to rely on exceptional circumstances pathways. For PWS, several medicines are currently being investigated internationally, and treatment options are evolving. However, because New Zealand is a small market, and because rare disorder medicines are often expensive, there may be significant delays before new treatments become routinely funded through standard pathways. The ECF and NPPA process therefore remain important for people living with rare disorders.

We encourage Pharmac to reconsider the current Factors of Consideration assessment approach to ensure it adequately reflects the realities of rare disorders. Medicines for rare conditions often have higher costs and smaller patient populations by their nature. Assessment processes that place significant weight on population-level impacts, cost offsets, or large patient numbers can unintentionally disadvantage people with rare disorders and undermine equitable access. Approaches more suitable for assessing rare disorder treatments are needed in this framework.

We also encourage Pharmac to consider how specialist expertise in rare disorders is incorporated into decision-making. New Zealand often has limited clinical expertise in rare conditions, and the experience of families is that they need to become the 'expert' in PWS and sometimes educate their clinicians. Where local expertise is unavailable or limited, there should be clear mechanisms for obtaining advice from recognised international experts and considering international standards of care.

Finally, we note the recent adoption of the Aotearoa New Zealand Rare Disorders Strategy, which recognises the need for more equitable and responsive health services for people living with rare disorders. We also welcome recommendations from the 2022 Pharmac Review. The outcomes of the review of this Exceptional Circumstance Framework should align with the objectives and expectations of the Rare Disorders Strategy and help ensure that people living with rare disorders can access appropriate treatment regardless of the rarity of their condition.

In summary, PWSA(NZ) supports the review and encourages Pharmac to:

- Develop clearer and more transparent guidance on the Exceptional Circumstances Framework and NPPA process, or an alternative similar pathway that Pharmac chooses to develop.
- Improve awareness of the framework among both clinicians and consumers.
- Ensure rare disorders are explicitly considered within future policy settings.
- Strengthen how equity is reflected in decision-making for people with rare disorders.
- Ensure access to appropriate rare disorder expertise when assessing applications.
- Align any future changes with the Aotearoa New Zealand Rare Disorders Strategy.