PHARMAC REVIEW
Te Arotake i Te Pātaka Whaioranga

New Zealand Government

Submission form

To help us to consider your submission we are asking that you focus on the following questions. There is the opportunity to provide additional feedback at the end. We expect to get a high response and ask that, where you can, you are concise. Once you have completed your submission please send it to: pharmacreview@health.govt.nz

Note that submissions are subject to the Official Information Act and may, therefore, be released in part or full.

If your submission contains any confidential information please state this within submission, and set out clearly which parts you consider should be withheld and the grounds under the Official Information Act 1982 that you believe apply. We will consult with submitters when responding to requests under the Official Information Act.

Submission questions

Tell us about your current experience with PHARMAC and how it functions

1. What is your understanding of what PHARMAC does? PHARMAC liaises with pharmaceutical companies to purchase and supply medicines for New Zealanders. They make decisions about which medicines to fund or decline and decide what priority a medication will be given for funding. In some circumstances, they decide the criteria a patient will need to meet to receive a funded medication. PHARMAC maintains various subcommittees that are involved in decision making processes.
2. What has been your experience of working with PHARMAC? The PWSA(NZ) has submitted to PHARMAC on several occasions to gain access to, and then widen access to, the only treatment currently available for people with Prader-Willi Syndrome. This treatment was available in other developed countries from the year 2000, on the basis of having a PWS diagnosis alone. In New Zealand, children had to wait until 2006 for funded treatment, but this was not on diagnosis basis and due to the restrictive criteria imposed, only a small minority of patients qualified for treatment. After further campaigning, many of these criteria were removed in 2009 allowing more children to qualify, although treatment still began at a later age (causing children to miss out on the essential benefits of earlier treatment) and was withdrawn at a younger age than in other countries. A few patients were still excluded. The PWSA(NZ), and our members, continued to campaign for widened access and the starting age for treatment was finally brought forward and additional restrictive criteria removed in 2017. Today, in 2021, this treatment is still not funded to the same level as overseas, with funded treatment ending at a significantly younger age.

The PWSA(NZ) experience of campaigning for this treatment for over 20 years has been extremely frustrating, especially as it has long been widely acknowledged to be a safe and life-changing medication for people with PWS by international experts. International consensus guidelines were produced by 43 international experts who recommended this treatment for all patients with PWS, for as long as benefits continue.

Over the years, the PWSA(NZ) campaign for funded treatment has involved submissions, parliamentary petitions, media campaigns and meetings with PHARMAC. Families have devoted much time and effort to this campaign, and some families fundraised in order to self-fund treatment. In more recent years, the cost of this treatment has greatly reduced enabling financially able families to self-fund continuation of this treatment for their child into adulthood. Access to treatment should not be wealth dependent and families should not have to consider this when they are already managing so many other issues and having a child with PWS is likely to have already financially impacted their lives.

One of the main obstacles we have found when working with PHARMAC is their baffling disregard and rejection of credible, convincing evidence. Our submissions have each referenced large bodies of research in support of the treatment requested, but we have received responses that suggest there is a lack of evidence. Sometimes we have been told that studies need to involve a higher number of patients – something that is difficult to achieve in a rare disease community. We have also been told that more longitudinal studies are required or that more new research to prove a particular benefit is required. However, as time goes on, there are fewer new studies because it is already widely acknowledged to be a very, effective treatment with multiple benefits. It is also unlikely that there will be further placebo-controlled trials as it would be unethical at this time to not provide this treatment to patients. PHARMAC have said that a new submission needs to contain new evidence, so this leaves us waiting for new trials which may never happen or are likely to be regarded as not robust enough. This is all quite baffling when other countries have seemingly reviewed the same body of evidence and could quite clearly see the benefits of this treatment long ago. This leaves us confused and with feelings that there has been a lack of transparency in PHARMAC's decision making in our case. We felt that there must have been other factors affecting decision making, perhaps they were purely monetary, but we also suspect that our treatment may not have been considered on its own merit and that acknowledging specific benefits for PWS patients may have been seen as a precursor to other groups seeking the same benefit. We also feel that there may be a lack of knowledge, awareness and understanding at a clinical advisory level.

Another comment regarding our experience of working with PHARMAC is that the reviewing of submissions and appeals has been an administratively slow process which is distressing for families. We discovered during one meeting with PHARMAC that our previous submission had not even been put before the endocrinology sub-committee. Administrative errors can happen and PHARMAC were apologetic and allowed this submission to be reviewed by the next PTAC meeting, but this was incredibly disappointing considering the huge amount of time and effort that goes into reviewing research, gathering support statements and putting a submission together.

3. What are the challenges with PHARMAC's functions for funding medicines and devices?

PHARMAC does not equitably assess or fund medicines for rare disorder groups and is not financially equipped to fund the many new, modern medicines that will become available. It is smaller user groups for whom medications are usually of higher cost who will continue to be disadvantaged.

What do you know about PHARMAC's processes and how they work?

4. What do you think works well with the processes PHARMAC uses to assess the funding of medicines and medical devices?

PHARMAC has been able to negotiate reduced cost medications and these cost savings result in being able to supply a reasonable number of low-cost medications to large population groups.

5. What do you think are the barriers to accessing medicines and devices?

No separate assessment criteria for rare disorder groups.

- Where fewer people need a medication, there will by nature be a higher cost – no bulk buying power, higher production cost, high research costs, sole supplier etc. The higher cost affects decision making, which does not provide equitable access for rare disorder groups.
- No allowance appears to be made for how clinical trials in rare disorder groups may differ, with lower participation levels due to smaller population size.
- Higher prioritisation appears to be given to 'providing the best outcomes for all New Zealanders'. This is a barrier to rare disorder groups accessing medicines because fewer people need access to the treatment and are therefore given less weight in decision making.

Lack of syndrome specific clinical knowledge, expertise and experience on decision making committees. Committees do not look outside New Zealand for expert opinion.

Rare disorders are not specifically acknowledged or formerly defined in health system policy.

A short-sighted approach to funding medicines and devices that does not appear to examine wider impacts on the health, mental health, and disability support systems. The current treatment for PWS and future pipeline treatments for PWS have life-changing outcomes that can reduce burden of both the health and care system.

6. Is there any other country that does it better? What is it that it does better and would any of those systems apply here?

Many other countries have systems where rare disorders are recognised as having complex needs requiring separate assessment pathways. Good examples can be found in Australia (Life Saving Drugs Programme) and the UK (NICE and PACE).

What should PHARMAC's role include in the future?

7. How might PHARMAC look in the future? And what needs to change for this to happen?

- Separate assessment criteria for rare disorders.
- Assessment criteria to consider the wider impact on the health and care systems.
- Assessment criteria to consider the wider impact on families and the community (i.e. education, disability support, mental health of families.)
- Assessment criteria to value and include the input of patient, family and community voice. Parents of children with PWS and patient advocacy groups like ourselves are often highly knowledgeable about who we support and their needs. Parents will spend countless hours reading information and researching – they will often inform health professionals that they see.
- A fast-track or rapid-access pathway for life saving /altering medications.
- The seeking out of relevant expertise when assessing the effectiveness of medications, and to utilise expertise overseas if necessary.
- Checks that panel members reviewing a medication have the appropriate up-to-date knowledge and expertise.
- Access to ongoing training for doctors in rare disorders, such as PWS.
- Improved transparency.
- Government funding needs to be examined in terms of how best to ring-fence funds and calculate need (and future need) for rare disorder groups and high-cost medications.
- There seems to be issues with PHARMAC not asking for additional funds when needed and governments not questioning this. This appears to stem from concerns surrounding negatively influencing PHARMAC's buying power – but at what cost to patients? There needs to be a shift in priorities, and responsibility should fall on both PHARMAC and governments to monitor medicine funding levels and ensure they are in alignment with need.

8. Are there additional or different things that PHARMAC should be doing?

- The collection of data for a registry of patients with rare disorders. (This needs to happen in conjunction with Health.)
- Promotion of research.

9. What do the wider changes to the Health and Disability system mean for PHARMAC?

Wider health system reforms need to reflect the complex needs of rare disorder patients. Rare disorders are not formerly defined or catered for in health system policy. There is a need for a clear framework for rare disorders which allows for coordinated medicines, health, education and disability system support.

How should PHARMAC address the need for greater equity in the decisions it takes, in particular for Māori, Pacific and disabled people?

10. How well does PHARMAC reflect the principles of Te Tiriti o Waitangi?
11. How can PHARMAC achieve more equitable outcomes? PHARMAC needs to be more inclusive of the needs of rare disorder groups. Detail has already been provided above.

Additional feedback

Is there anything else that you think the Review Panel should consider? Systemic change is needed in the processes that PHARMAC use, but although government funding is not within scope of this review, it cannot be ignored that funding levels remain woefully short of what is needed, and that we consistently lag far behind other OECD countries in the funding of medications. When medications are declined that are widely available overseas, patients and families are left feeling that their lives and well-being are not considered important enough.

Contact information

Your feedback is important to us. If you are comfortable for us to get in touch if we have any questions or points of clarification regarding your feedback, please provide your name and contact email address below.

Name	Jo Davies, Operations Manager
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Organisation	Prader-Willi Syndrome Association New Zealand

If you do not want your personal details to be shared for any other purpose (for example if we receive a request for information under the Official Information Act) please signal this using the box below.

☐ I do not want my personal details to be shared for any purpose other than this review.

Thank you for providing your feedback.

Tēnā koe mō tō tuku urupare mai.