Submission to Pharmac on proposal to decline inactive funding applications

To: applicationfeedback@pharmac.govt.nz
Date of Submission: 8 February 2024
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Declaration of interest

Rare Disorders NZ works with clinicians, researchers, allied health professionals, academia, government and industry to achieve better outcomes for people with a rare disorder in New Zealand and their whānau. We are funded by grants, donations, fundraising events, Pharma roundtable and a small Te Whatu Ora contract. This submission is in response to Pharmac's proposal to decline inactive funding applications (released 18 December 2023).

Rare Disorders NZ

Rare Disorders New Zealand is the respected voice of rare disorders in Aotearoa. We are the national peak body organisation, supporting the 300 000 New Zealanders with rare disorders and the people who care for them. We help those affected by rare disorders navigate the healthcare system, find information and resources, and connect with support groups specific to their condition.

We proudly advocate for public health policy and a future healthcare system that works for those with rare disorders – using a strong and unified voice to collaborate with Government, clinicians, researchers, and industry experts, to promote diagnosis, treatment, services, and research.

Our vision is for New Zealand to become a country where people living with a rare disorder are fully recognised and supported with equitable access to health and social care.

Our submission

Thank you for the opportunity to respond to this proposal that includes the potential decline of multiple inactive funding applications for medicines for people with rare disorders.

Rare Disorders NZ is pleased to see Pharmac aiming to provide more clarity about which medicines they are actively considering for funding. We welcome any additional transparency and efficiency from Pharmac.

We agree that it is important people have certainty about whether a medicine is, or is not, being considered, even if Pharmac's decision is to decline funding.

Along with making it clear which applications are no longer being considered, Rare Disorders NZ urges Pharmac to have processes that ensure that individuals and groups who make submissions throughout the application process are notified of any decisions, further consultation opportunities and outcomes.

We are concerned to see that the last date many of these applications were considered by clinical advisors was some time ago and that there does not appear to be provisions for consideration of new evidence prior to the final decision to decline these applications being progressed.

Regarding the specific inactive funding applications being considered for decline, we have consulted with some of our support group leads and have comments or oppose the decline of the following specific applications:

- **Application for Alglucosidase alfa for late onset Pompe disease**

Alglucosidase alfa is the only medicine Medsafe approved for Pompe at this time. There is another second generation medicine (Nexviazyme) currently going through the Medsafe approval process. While it is disappointing to see the decline of this application proposed, and we would love to see it progressed, the transparency is appreciated.

The Pompe support group are seeking assurances that the decline of Alglucosidase alfa for late onset Pompe disease will not adversely impact any potential Pharmac assessment of Nexviazyme or other future medicine, including that the decline would mean there aren't comparison options available when looking at cost.

- **Application for Bevacizumab for second-line treatment of high-risk advanced ovarian cancer**

We are frustrated and disappointed to see this application proposed to be declined.

The group Cure our Ovarian Cancer report that rare ovarian cancers have no specifically approved treatment therapies in New Zealand and are known to have poor response rates to Pharmac funded chemotherapy options, particularly in recurrence (often 20% or less).

Recent research (Lucia Musacchio et al., *Effect of bevacizumab in advanced low grade serous ovarian cancer: Data from the MITO 22 trial;* Kylie Gorringe, *Targeted therapy for mucinous ovarian carcinoma: evidence from clinical trials*, 2022; and Toshiyuki Seki *Bevacizumab in first-line chemotherapy to improve the survival outcome for advanced ovarian clear cell carcinoma: A multicenter, retrospective analysis*, 2022) highlights the significant benefit of bevacizumab in rare ovarian cancers in shrinking tumours and extending progression free survival - improving quality of life when cancer growth is symptomatic and giving women precious extra time free from chemotherapy, which is significant in the context that chemotherapy often has little effect with significant side effects in this recurrent rare cancer population. Seki's paper also found a 16.7 month improvement in overall survival with the addition of bevacizumab.

One of the challenges with rare ovarian cancers is that there is little research, and little funding for research which limits the quality of evidence that Pharmac has for decision making. In that context the findings from the studies quoted are significant and we implore Pharmac to reconsider their decision.

Rare Disorders NZ supports Cure Our Ovarian Cancer's call to reconsider funding Bevacizumab for second-line treatment of high-risk advanced ovarian cancer.

- **Application for Sapropterin for Hyperphenylalaninaemia due to phenylketonuria (PKU) in non-pregnant PKU patients**

The PKU New Zealand group are aware of upcoming treatments that are more likely to work for a larger proportion or even the whole population, but feel that until such a time this is the reality, Sapropterin should be an option that is available, especially if other options in the future don't suit or aren't tolerated well.

We are concerned about the false equivalence formed by PTAC in 2016 where: "*Members noted that in general, as individuals with PKU age, adherence to the diet decreases (Koch et al. J Inherit Metab Dis 2002;25:333–46). The Committee questioned whether patients would adhere to medication, if they did not adhere to dietary restrictions*".

The PKU group note that taking medicines is easy, especially for their population who had bland, or even nasty tasting medications forced into them from a young age. In comparison,

maintaining a heavily restricted diet lifestyle when juggling work and/or study, social commitments and family is difficult to maintain.

Rare Disorders NZ urges Pharmac to consider the lived experience voice in such situations and that a decision to decline this application is not made until the lived experience perspective is incorporated into further assessment.

We would like to highlight that PTAC declined this application in 2016. Given that we are now 8 years on, we would like to see consideration of new research, and the possibility of adding in genetic testing for those most likely to respond. This could help strengthen the case and take away some of fiscal risk discussed by PTAC in 2016 associated with funding sapropterin for the entire PKU population.

We also urge Pharmac to consider any evidence that emerges from Australia's experience, as they have recently approved Sapropterin/Kuvan use, prior to declining this application.

ENDS

Rare Disorders NZ

8 February 2024