



Re: Proposal to fund supplements for phenylketonuria and other inherited metabolic disease

To: consult@pharmac.govt.nz

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Submitted by: Rare Disorders NZ

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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 fund to fund supplements for phenylketonuria and other inherited metabolic disease.

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

Rare Disorders NZ welcomes Pharmac expanding the range of supplements available to provide greater choice and improve the lives of people with Phenylketonuria (PKU) and other inherited metabolic disease. However, we also note that while New Zealand is making steps towards meeting internationally recognised standards of care, there is still a way to go in terms of both medication and food/supplement access for those with inherited metabolic disease.

We have heard the following from members of our community with PKU:

- We hope this expansion spearheads further funding, especially for more variety of low protein food products, not just supplements. It is important to have a variety of options for this diet, and we believe that this would also help more people to stay on the diet.
- Increasing the variety of options available allows those who are not satisfied with the current medicines and foods to try new things. We believe that ultimately this increased variety could result in better dietary adherence, or for those returning to diet, it could allow for an easier time with the chance to try several different options and find something more palatable than what was available when they were younger.
- The problem we have, as with other rare disorders in New Zealand, is keeping up with the newer treatments/medications/foods available around the world that greatly improve the quality of life for individuals with these rare disorders, in our case PKU.

Rare Disorders NZ is pleased to see Pharmac recognise that the current NPPA processes create administrative burden for healthcare professionals and uncertainty for both prescribers and patients. We appreciate that Pharmac is addressing this and hope to see more rare disorder medicines listed rather than only be accessible via NPPA.

ENDS

Rare Disorders NZ

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