Re: Proposal to fund nitisinone for people with rare inherited metabolic disorders

To: Consult@Pharmac.govt.nz
Date of Submission: 11 December 2025
Submitted by: Rare Disorders NZ
Contact person: Chris Higgins (CE)
Email: Chris@raredisorders.org.nz

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 the Proposal to fund nitisinone for people with rare inherited metabolic disorders

Rare Disorders NZ

Rare Disorders New Zealand (RDNZ) 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 living with rare disorders to navigate the healthcare system, find information and resources, and connect with support groups specific to their condition.

Our vision is for New Zealand/Aotearoa to become a country where people and whānau living with a rare disorder experience best possible health and wellbeing. We seek to achieve this by enabling and empowering people with rare disorders to best benefit from services and therapies available in New Zealand, and by championing their collective voice, advocating for provision of world leading evidence based health, disability, education and other services.

A rare disorder is a medical condition with a specific pattern of clinical signs, symptoms and findings that affects fewer than or equal to 1 in 2,000 people in Aotearoa New Zealand. Rare disorders include, but are not limited to, rare conditions among genetic disorders, cancers, infectious disorders, poisonings, immune-related disorders, idiopathic disorders and various other rare undetermined conditions. An ultra-rare disorder is a medical condition with a specific pattern of clinical signs, symptoms and findings that affects fewer than or equal to 1 in 50,000 people in Aotearoa New Zealand

Submission

Rare Disorders NZ supports nitisinone being listed on the Pharmaceutical Schedule and funded for people in New Zealand who need it. We note that nitisinone is currently funded for people with tyrosinemia type 1 or alkaptonuria, two rare inherited metabolic disorders, through Pharmac's exceptional circumstances framework (NPPA).

It is positive to see Pharmac making changes to processes that reduce the administrative burden for health care professionals, provide transparency around criteria for access and provide certainty of ongoing supply for the people who need it.

We are pleased to see flexibility built into this proposal with an Alternative Brand Allowance proposed to be available for people who are unable to change to the Nitisinone (LogixX Pharma) brand of nitsinone capsules for clinical reasons.

Rare Disorders NZ encourages Pharmac to ensure adequate consultation has occurred with clinicians who have expert knowledge in these inherited metabolic disorders and who prescribe nitisinone to ensure that the changes being made meet their and their patients' needs.

While this change is welcome, we note that this is not a case of a new medicine being funded for rare disorders, simply a shift in the process to obtain an already funded medicine.