Submission to Pharmac on Proposal to fund Risdiplam (Evrysdi) for Spinal Muscular Atrophy

To: consult@pharmac.govt.nz
Date of Submission: 24 February 2023
Submitted by: Rare Disorders NZ
Contact person: Michelle Arrowsmith (CEO)
Email: michelle@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 that is due to end June 2023. This submission is in response to Pharmac's proposal to fund Risdiplam (Evrysdi) for Spinal Muscular Atrophy in New Zealand.

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. All childhood cancers are rare.
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 have been long-time advocates for access to medicines for Rare Disorders including Spinal Muscular Atrophy (SMA).

It is important to reference the advocacy as it has taken a significant toll on patients, their loved ones, and the rare disorder community in the time that it has taken to get to this point. This is important to note as we would like this consultation process to consider the unmet need of many in the community that this proposal leaves behind. We would like to see changes to include the full community so the relentless advocacy efforts can be focused on other areas of unmet need within the rare disorders community as opposed to access to medicine.

Rare Disorders NZ fully support the consultation and funding of this drug/medicine as outlined in the consultation proposal.

Rare Disorders NZ supports the funding proposal for Risdiplam which we understand would have the same access criteria as nusinersen (Spinraza) for the treatment of symptomatic type 1, 2 and 3a SMA for people who start treatment when they are 18 years or younger.

Rare Disorders NZ supports two funded options for the treatment of symptomatic SMA in New Zealand, especially as Risdiplam would provide an oral treatment option alongside nusinersen.

Rare Disorders NZ reinforces Pharmac's estimation of the numbers of people who are likely to be supported by mediciation access for SMA in NZ. "SMA is a rare disorder. We estimate that in the first year, 30 to 50 people would be eligible for funded treatment with either nusinersen or risdiplam. We expect that each year up to four people may be diagnosed with SMA and be eligible for treatment. As a result, the number of people receiving treatment is anticipated to increase over time."

Rare Disorders NZ would like to see that Risdiplam is funded in the pre-symptomatic setting if it is approved by Medsafe for this use in New Zealand. Rare Disorders NZ looks forward to seeing the outcome of the consideration of Risdiplam in the pre-symptomatic setting being considered by the Rare Disorders Advisory Committee in March 2023. We urge the Rare Disorders Advisory Committee to support this pre-symptomatic setting and not delay access further to those with SMA in NZ.

Rare Disorders NZ supports a process to be set up to manage the supply of risdiplam to an individual's location that is supportive, flexible and listens to the needs of the person and their whanau who is requiring this medication, and where appropriate this supports local flexible options and options for self-administration.

Rare Disorders NZ supports the views Pharmac has heard that people would like wider access to SMA treatments, including for those who are aged over 18 years at the time they start treatment. Rare Disorders NZ believes no one should be left behind, and that this is a matter of equity. We urge the Rare Disorders Advisory Committee to support this widening access and not delay access and inequities further to those with SMA in NZ.

Support for Submission by Fiona Tolich

Rare Disorders NZ also supports the submission written by Fiona Tolich, Patient/Patient Advocate/Trustee Patient Voice Aotearoa/Lead Advocate SMA NZ. Fiona's submission is included below;

"I am writing this letter in support of the proposal to fund Risdiplam for Spinal Muscular Atrophy.

With what has happened over the last week, it has highlighted truly how vulnerable we are when it comes to accessing treatment, on a different level to what I would typically talk to.

Having access to an oral therapy for the families in cyclone impacted areas would have taken a major load off. They were able to start due to compassionate supply and there were posts shared in the SMA community highlighting just how special this was. We cannot take that hope and access away from others whose journey is yet to begin.

To have access to a treatment for this gruelling rare disease in the middle of a National State of Emergency is a good reason to have the option of a self-administered medicine. I cannot imagine how these families would have navigated anything else at this time when they have lost so much.

I take note that you have referenced including pre-symptomatic if the Rare Disorders Advisory Committee recommend this in March and following Medsafe approval. I look forward to hearing positive news there.

I also note that there is further advice being sought around the widening of access to adults and additional subtypes. I feel strongly that no person living with SMA should be left behind. Treatment is so important, which is why I have moved countries. I would like to see Pharmac bringing in those with lived experience and a neurologist from Australia who is treating patients in these wider groups. It is crucial that experts in treatment provide accurate insight into why this must be expanded.

In terms of delivery, it would be great if there was an option based on their personal circumstances to determine if a direct to home or delivery to a different (local) location is their preference.

We as a community look forward to hearing a positive outcome and further announcements in terms of widening access so no one is left behind.

Regards

Fiona Tolich
Patient/Patient Advocate/Trustee Patient Voice Aotearoa/Lead Advocate SMA NZ"

ENDS

Rare Disorders NZ

24 February 2023