PO Box 7081
Newtown
Wellington 6242
027 2500619
www.raredisorders.org.nz

12 June 2025

Hon David Seymour
Associate Minister of Health (Pharmac)
D.Seymour@ministers.govt.nz

Dear Minister,

Thank you again for your recent attendance at our Rare Disorders Parliamentary event, which focused on improving diagnosis and medicine access for individuals living with rare disorders. Your presence and engagement were greatly appreciated by our community.

I am writing ahead of your anticipated 2025/2026 Letter of Expectations to Pharmac. We were particularly encouraged by Point 18 in your previous Letter of Expectations, which directed Pharmac to work with the Ministry of Health during the development of the Rare Disorders Strategy. This recognition of the importance of rare disorders was a significant and welcome step for our community.

As the Rare Disorder Strategy moves from development to implementation, we ask that the upcoming Letter of Expectations include a similar directive that Pharmac develop concrete plans for actioning the priorities outlined in the Rare Disorders Strategy. We hope this would include consideration of options to provide equitable access to modern rare disorder medicines through a specific assessment pathway. We also request that these plans, along with progress updates, be included in Pharmac's performance and annual reporting to ensure transparency and accountability.

In your prior Letter of Expectations, you also referenced the 2022 Pharmac Review's findings around the need for improvements in transparency and timeliness. Timeliness remains a particular concern for rare disorder medicines. For your reference, we have attached a table highlighting a selection of rare disorder medicine applications that have stalled at the assessment phase within Pharmac, despite having received high priority recommendations from clinical advisory committees. We hope to see continued emphasis in your expectations that timely assessment of such applications is improved.

Thank you for your continued support of those affected by rare disorders in New Zealand. We would welcome the opportunity to discuss this further.

Warm regards,

Chris Higgins
Chief Executive
Rare Disorders NZ

Rare disorder medicines that are awaiting assessment having received a high priority recommendation			
Pharmaceutical	**Requested for**	**Clinical advice last received**	**Recommendation**
Ivacaftor (Kalydeco)	Cystic fibrosis, mutations responsive to ivacaftor (Application made to widen access to the 97 US FDA approved CF mutations)	27 April 2022 (Respiratory Advisory Committee)	High Priority
SMA treatments (nusinersen (Spinraza) and risdiplam (Evrysdi))	People with SMA type II and III who are aged 19 years and over	7 March 2023 (Rare Disorders Advisory Committee)	High Priority
Elosulfase alfa	MPS IVA for children under the age of two years	7 March 2023 (Rare Disorders Advisory Committee)	High Priority
Lanadelumab (TAKHZYRO®)	Routine prevention of recurrent attacks of hereditary angioedema	29 May 2024 (Rare Disorders Advisory Committee)	High Priority
Rituximab (Rixiymo)	Systemic sclerosis with interstitial lung disease (ILD), not responsive or intolerant to mycophenolate mofetil	28 August 2024 (Respiratory Advisory Committee)	High Priority
Elexacaftor/tezacaftor/ivacaftor and ivacaftor (Trikafta)	Cystic fibrosis, aged 2-5 years, with F508del mutation(s) in the CFTR gene, G551D mutation or other mutation responsive in vitro to Trikafta	28 August 2024 (Respiratory Advisory Committee)	High Priority
Blinatumomab (Blinctyo)	Measurable residual disease in B cell lineage acute lymphoblastic leukaemia	10 October 2024 (CTAC)	High Priority