Re: Therapeutic Products Act Repeal Bill

To: Health Committee he@parliament.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 the Therapeutic Products Act Repeal Bill.

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.

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.

Our submission

Rare Disorders NZ (RDNZ) acknowledges the significance of the Therapeutic Products Act (the Act) in aiming to regulate therapeutic products comprehensively, balancing the associated risks and benefits. While we supported the Act's objectives, we had certain concerns with the Act as passed. We are very interested in the promised "better regime" that Hon Casey Costello has signalled will be put in place, and we see the repeal as an opportunity to do better for the rare disorder community.

The current processes for, and access to, medicines for those living with rare disorders in New Zealand are already significantly behind other countries, including Australia, the UK, and Europe. We were concerned that the Act would put such conditions into the system that would make it even less attractive than now for companies to sponsor their products into New Zealand. We hope this repeal is the first step in creating a new regime that:

- Has specific considerations for the unique nature of rare disorder medicines.
- Provides for an orphan drug registration scheme similar to Australia's TGA to support medicines and therapies for rare disorders.

RDNZ is clear, through evidence and international practice, that types of biological innovation are where medical technology is delivering for those living with rare disorders. We had grave concerns that the Act did not have sufficient detail to allow flexibility to ensure a balance between regulation and safety, new technologies, and innovation- especially for rare disorder therapies.

We were particularly worried that the Act would cause delays in regulating and implementing new technologies for rare disorders here in New Zealand. We look forward to this repeal leading to a new regime that supports improvements in this area and expedites access to new technologies. The new regime must align with international best practice and be future-proofed so that new and emerging health technologies can be regulated appropriately and without undue delay.

Throughout the reading of the Therapeutic Products Bill, there was a lot of commentary around its effect on the ability of patients to access unauthorised medicines and compassionate access schemes. The repeal of the Act temporarily puts our community's minds at ease in regard to this matter. We ask that work on any future regime carefully considers how to clearly allow access to unauthorised medicines and compassionate access schemes without creating additional administrative or financial burden to any involved parties.

Rare Disorders NZ was also concerned that the Act would result in the clinical trial framework being moved to the Therapeutic Products Regulator, with the loss of the currently legislated 45-day timeframe within which a decision has to be made on the application. It seemed likely this would lead to delays in clinical trials reaching New Zealand and create a barrier to the access of new medicines here. With specific regard to rare disorders, we were concerned that the potential increased uncertainty, financial, and administrative burden placed upon companies would make New Zealand an unappealing location for conducting a trial.

We were pleased to see some amendments in the Therapeutic Products Bill as it progressed to the Act, namely in relation to advertising provisions and the importation of prescription medicines. We hope to see what was learned in these areas carried over to the proposed new regime following this repeal.

Rare Disorders NZ looks forward to engaging in the development of the new regime following this repeal, and we are pleased to hear that timely access to new and promising therapies will be a priority for this proposed system.

ENDS