Submission to Parliament on the Therapeutic Products Bill

To: Health Select Committee via parliament website submission
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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 that is due to end June 2023. This submission is in response to the proposed Therapeutic Products Bill 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

We wish to make the following comments.

1. Innovation balance between regulation and safety

The purpose of the Bill is to "protect, promote, and improve the health of all New Zealanders by providing for the: acceptable safety, quality, and efficacy of medicines;

acceptable safety, quality, and performance of medical devices; acceptable safety and quality of natural health products, and that any health benefit claims are supported by scientific or traditional evidence."

"Both medical devices and gene, cell and tissue therapies are currently not fully regulated in New Zealand so the Bill seeks to address this through a risk-proportionate approach. Pre-market controls will provide assurances that products are made to the proper standards, while in-market and post-market controls will enable a timely response if safety issues arise."

Rare Disorders NZ (RDNZ) has grave concerns around the flexibility of this Bill and its processes, of which much if not all is yet to be detailed out sufficiently. We are concerned about the risk-proportionate approval systems to ensure New Zealanders especially those living with a rare disorder can access necessary life-saving medicines. Currently most people living with a rare disorder in NZ with a suitable medicine to treat or cure their rare disorder cannot access such medicines in NZ. We do not see any assurances that this Bill seeks to address this situation, in fact we have concerns that this Bill will likely worsen the situation.

The Bill seeks to ensure the new regime will align with international best practice and it will be future-proofed so that new and emerging health technologies can be regulated appropriately. RDNZ is clear though evidence and international practice that types of biological innovation are where medical technology is delivering for those living with rare disorders. We have grave concerns that there isn't sufficient detail to allow flexibility to ensure a balance between regulation and safety and new technologies and innovation. We have real concerns that this Bill will cause delays in regulating and implementing new technologies for rare disorder here in NZ. NZ is already behind the UK and Europe in these medicine and gene therapy advances. NZ currently isn't even able to regulate, provide appropriate technology assessments and fund and implement drug access for rare disorders. We cannot see how this Bill support improvements in this area and supports the expediting of new technologies. The harm of waiting for full data must be weighed against potential harm for expedited approval with appropriate safeguards in place, as is international best practice.

2. Advertising and Advocacy

Rare Disorders NZ has grave concern regarding advertising and advocacy because clause 193 states not allowing some forms of advertising, and it says, "(1) An advertisement for a therapeutic product means a communication made for the purpose of promoting the product. (2) A communication means a communication made in any way whatsoever

(including, for example, by an individual in person, using a physical object, in print, or using any kind of information or communications technology)."

The Bill states you are not allowed to communicate about therapeutic products that aren't regulated in New Zealand already, and the only people who have carve-out are the Ministry of Health, Medsafe, and Pharmac.

This will have major impact for all patient advocacy groups including Rare Disorders NZ. Over the last few years and up to recently Rare Disorders NZ along with other patient advocacy groups have been advocating for Trikafta for cystic fibrosis, Spinraza for spinal muscular atrophy and ustekinumab for people with Crohn's disease and colitis. These types of medications weren't regulated in New Zealand or funded by Pharmac. It looks entirely possible that we along with others who are advocating, for their own lives and their own children's lives, for drugs that have been regulated overseas and not yet in New Zealand to find themselves in breach of this Bill/Act because they are communicating and advertising for their right to have drugs and products in New Zealand. We require the health select committee and the Government to ensure that this won't be the case by ensuring changes to this Bill. We believe if this doesn’t occur then potentially our rights for advocacy are being breached.

3. Cost Burden

This Bill provides no suitable replacement of section 29 form Medicines Act (1981). Sponsors will have to pay fees for licenses or permits. It is not clear how much fees will be and any exceptions to this rule, specifically for medicines and therapies for rare disorders.

The loss of with no replacement for section 29 creates an issue for Rare Disorders patients on a number of fronts;

A number of Rare Disorders medicines come into NZ via section 29. So does this mean the access to these drugs will cease and this available route with no alternative will cease to exists and therefore this prevents medicines for rare disorders entering the country in the future. Even Pharmac themselves uses section 29 for the NPPA system and for the rare disorders medicines via the the NPPA process.

There are a number of companies who are providing medicines under compassionate access (free to patients), the removal of section 29 and the new Bill requiring payment for license or permit will likely mean companies will be less inclined to provide free compassionate access to medicines for rare disorders in the future. This is often the only route New Zealanders can get access to life saving drugs, such as the current situation for those with Pompe Disease.

Rare Disorders NZ has concerns about whether the costs outweigh the benefits. The health select committee and Government need to make sure that this new Bill and formation of a new regulatory body does not impose such a heavy burden on a company that they don't actually want to come to New Zealand and regulate their products here. In the case of some medical technology medicines and devices, there might only be limited numbers required in New Zealand, and we want to make sure that we're not putting in place such a burden that companies decide to miss our NZ market completely and that all New Zealanders miss out. Rare Disorders NZ believes this is a very real risk for New Zealand.

4. Medicines for Rare Disorders

The current processes and access for medicines for those living with rare disorders in NZ is already significantly behind other countries, Australia, UK and Europe. We can only see that this bill would put such conditions into the system that would make it even less attractive than now for companies to sponsor their products into NZ. The new Bill/Act should have specific ability for an orphan drug registration scheme similar to Australia's TGA to support medicines and therapies for rare disorders.

5. Regulation

The bill states “the regulator will be a public servant appointed by the Director-General of Health on the basis of their relevant knowledge and expertise. The Regulator will exercise their powers under the Bill independently of the Director General of Health and the Minister of Health but may be subject to general policy directions issued by the Minister”. We suggest that the regulation section is reviewed to ensure this is as independent as possible and that it allows for an independent review to be requested by the regulator using the right national and international experts and evidence. We suggest that this is made more explicit and transparent in the bill to allow this to be executed in practice.

We wish to make the following recommendations.

RDNZ are concerned about the risk-proportionate approval systems to ensure New Zealanders especially those living with a rare disorder can access necessary life-saving medicines. We ask that there are specific allowances made within the Bill for medicines for rare disorders, that takes into account the risk benefit equation for medicines for rare disorders which is different to other medicines used for wider know conditions amongst larger groups of the population.

RDNZ ask that the Bill is amended to ensure there is carve out for advertising, advocacy and communication allowed by charities, not for profits and advocacy patient groups and individuals for all therapeutic products that aren't already regulated in New Zealand.

RDNZ ask that the Bill/Act is amended to ensure there is provision for an orphan drug registration scheme similar to Australia' s TGA where Medsafe would waive fees for medicines for rare disorders.

ENDS

Rare Disorders NZ

5 March 2023