Re: Medicines Amendment Bill

To: Committee Secretariat, Health Committee
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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 Medicines Amendment 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 welcomes the Government's commitment to improving access to medicines and supports key components of the Medicines Amendment Bill, including the introduction of a streamlined verification pathway and updated prescribing settings for unapproved medicines in appropriate clinical contexts.

However, we share the concerns raised by Medicines New Zealand about the effectiveness of the proposed "consent by verification" or "rule of two" mechanism in achieving the Bill's goal of improving access, particularly for people with rare disorders.

Many rare disorder therapies are approved internationally through expedited pathways, such as provisional or conditional approvals by regulators like the US FDA. These pathways are designed to address urgent and unmet clinical needs, and are often the only viable option for patients with rare and life-limiting conditions. Yet, the proposed verification pathway excludes these types of approvals as valid references, meaning New Zealand will lack a streamlined mechanism for accessing treatments already deemed urgently necessary overseas.

In addition, many rare disease therapies are reviewed through international collaborative initiatives (e.g. *Project Orbis*, *ACCESS Consortium*), which typically result in a single harmonised evaluation report rather than two separate assessments. Requiring two full evaluation reports makes it extremely difficult, or impossible, for these therapies to qualify for verification, disproportionately disadvantaging rare disorder treatments, which are often developed via these very processes to accelerate global access.

We are also concerned that the Bill recognises the US FDA's Centre for Drug Evaluation and Research (CDER), but not its Centre for Biologics Evaluation and Research (CBER), which regulates advanced biological and gene therapies. Many rare disorder treatments fall into this category, including gene, cell, and tissue-based therapies. These advanced therapy medicinal products (ATMPs) are increasingly common internationally and need to be incorporated into this Bill to ensure it is future-proofed.

Rare Disorders NZ also supports Medicines New Zealand's position in opposing the proposed shift from calendar to working days for statutory timeframes under Section 24 (Changed Medicine Notification applications) and Section 30 (Clinical Trials Approvals). If statutory timeframes are to be expressed in working days, the number of days should be reduced accordingly to preserve current timelines.

Extending approval timelines (from 45 calendar days to 45 working days) will delay clinical trial approvals, making New Zealand less attractive for research investment and further limiting access to clinical trials for people with rare disorders. With many studies

involving competitive recruitment, such delays risk New Zealand patients losing access to participation opportunities in favour of countries with faster approval systems.

People living with rare disorders need a regulatory system that reflects how their therapies are developed and approved internationally, as well as that they are small patient populations who often face different risk–benefit considerations, especially when no alternative treatment options exist. The needs of people living with rare disorders have been largely overlooked in the current Bill, despite the government's Rare Disorders Strategy[i], which commits to ensuring that rare disorder communities are considered in all system changes and priorities.

Rare Disorders NZ urges the Select Committee to ensure that this Bill meaningfully provides streamlined access to modern, life-changing therapies for the rare disorder community, and does not damage their access to clinical trials now and in the future.

We ask that the Select Committee honour the Rare Disorders Strategy and design a streamlined verification pathway that accommodates the unique nature of rare disorder therapies through:

1. Ensuring that provisional/conditional approvals for rare disorder therapies from trusted international regulators can be used as reference approvals under the verification pathway.
2. Expanding the list of recognised regulatory authorities to include CBER (FDA).
3. Accounting for global collaborative review processes for rare disorder therapies and how these can be utilised instead of requiring two full evaluation reports.
4. Enabling reliance-based decision-making for ATMPs to improve regulatory efficiency and future-proof the Bill.
5. Reconsidering the change to working days in statutory timeframes for Sections 24 and 30 or adjusting timelines to preserve current efficiency.
6. Ensuring transparency and stakeholder engagement in the development of secondary legislation, especially in regard to the regulation of advanced therapies.

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[i] Ministry of Health. 2024. Te Rautaki o Aotearoa e Pā ana ki ngā Mate Mokorea - Aotearoa New Zealand Rare Disorders Strategy. Wellington: Ministry of Health.