Draft Submission to Pharmac's review of rule 8.1b of the Pharmaceutical Schedule

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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 review of rule 8.1b of the Pharmaceutical Schedule and the funding of paediatric cancer treatments 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.

Question 1

Is our understanding of the overall health outcomes being achieved for people with paediatric cancers correct? If not, please provide any further information or context.

The overall childhood cancer survival statistics listed in Tables 3, 4 and 5 of the Pharmac discussion paper *Funding of paediatric cancer treatments in New Zealand* reports a 5-year survival rate of 86% across all childhood cancers. However this statistic masks the relatively dire survival rate for CNS tumours which have a 5-year survival of just 73.5% and are responsible for 42% of all cancer deaths in children aged 0-14 years[1].

The 5-year survival rate of 73.5% is averaged across all brain tumour types and misrepresents the real world situation for the aggressive sub-types of paediatric brain and CNS tumours because:

1. The overall survival rate includes benign CNS tumours which have a much better prognosis and longer survival time than malignant CNS tumours;

[1] *Childhood cancer survival in Aotearoa, New Zealand 2010-2019*, National Child Cancer Network

2. The overall survival rate masks the survival data for aggressive sub-types such as DIPG/DMG (diffuse intrinsic pontine glioma/diffuse midline glioma) which has a 5-year survival of 2% and median overall survival of 8-11 months[2].

Furthermore, survival statistics fail to tell the whole story. They do not accurately describe the harm, suffering and multiple stresses experienced by families dealing with a paediatric cancer diagnosis, especially those with a rare cancer such as brain tumours.

The lack of psychosocial support systems in the community is a constant area of concern in the childhood cancer and rare disorders community, particularly immediately after discharge from hospital. Leaving hospital with a rare condition and returning home to a local community where people have no experience of caring for a child with cancer or a rare disorder generates feelings of isolation or not belonging.

The multiple stresses surrounding obtaining a correct diagnosis, access to imaging, and access to allied health services, including psychosocial support and palliative care services, impact the quality of life of both patients and their whānau.

Financial toxicity is a term used to describe financial hardship experienced by families having to self-fund medical treatments. This is not uncommon in families with a childhood cancer diagnosis, as the changing family dynamic often necessitates one or more parent changing their work situation to care for the patient. It is yet another stress for families who are already dealing with a serious medical diagnosis.

Any changes to rule 8.1b which restricts access to new medicines has the real potential to cause significant financial toxicity to families. These multiple stresses are not adequately represented in a mere survival statistic.

RDNZ believes due to a lack of knowledge as illustrated by Pharmac's questions in the consultation document, that Pharmac uses its powers under 69(1)(c) of the Pae Ora (Healthy Futures) Act 2022 to engage "in (independent peer reviewed) research to meet the objectives set out in section 68(1)(a) (of the Act), specifically to address the unknowns around removal of a tool that appears to support equality of outcome with regards to childhood cancers in Aotearoa and specifically for Māori and Pacifica tamariki.

Question2
In what other clinical contexts is participation in clinical trials the 'standard of care'?

Whilst national standards of care are not necessarily documented and available for rare disorders in New Zealand the participation in clinical trials is especially important for those with a rare disorder. This is often the only way to receive what is deemed internationally a known standard of care or is part of an international care pathway or treatment option.

Rare Disorders NZ believes that any disease with a poor prognosis and lack of effective known and prescribed treatments would benefit from 'participation in a clinical trial' as part of the standard of care, including childhood cancers and other rare disorders.

Question 3
To what extent is access to paediatric cancer clinical trials dependent on access to medicines through rule 8.1b?

[2] *Hoffman, L.M. et al. Clinical, radiological, and histo-genetic characteristics of long-term survivors of diffuse intrinsic pontine glioma: A collaborative report form the International and SIOP-E DIPG Registries. Neuro-Oncology 18, iii65-iii66 (2016)*

Paediatric cancers are rare. This makes drug development for these cancers difficult because:

1. Biotech and pharmaceutical companies are less incentivised to develop drugs for paediatric cancers as the commercial opportunity is limited;
2. Many trials consist of a combination of different drugs belonging to different pharmaceutical companies. These combination trials are difficult to set up as they require close co-operation between commercial competitors;
3. Clinical trials for paediatric cancers are difficult to recruit due to the small patient population, resulting in high drug development costs and lower profit margins for developers.

For these reasons, many paediatric cancer trials are 'investigator-led' trials, or trials conducted through co-operative trial networks, rather than pharma-sponsored trials. Without the financial support of the pharmaceutical companies that supply the treatments, trial investigators in New Zealand require access to experimental medicines through rule 8.1b.

Question 4
How sensitive is this system of care to changes to rule 8.1b?

Any changes to rule 8.1b which result in reduced access to new paediatric cancer medicines will threaten the viability of clinical trials which rely on this rule for access to new medicines.

Outside of clinical trials, restricted access to new medicines will prevent paediatric oncologists from prescribing novel treatments which:

1. Have shown efficacy in clinical trials but are yet to obtain final regulatory approval;
2. Have been approved by regulatory agencies (such as the United States' FDA or New Zealand's Medsafe) for other indications;
3. Have been approved by regulatory agencies (such as the US FDA, NZ's Medsafe, etc.) for the indication but are not funded by Pharmac.

Early intervention using new medicines is an important component of the system of care of paediatric cancers. Paediatric oncologists can use rule 8.1b to offer new treatments for patients who would otherwise have exhausted all other standard treatment options.

If rule 8.1b was changed so that new medicines were required to go through the regular Pharmac funding process, we believe the current system of care would be adversely affected as access to these medicines would be severely restricted.

Rare Disorders NZ believes that rule 8.1b is essential to the care system here in New Zealand. Without this rule in place New Zealand will go backwards in its care delivery and outcomes for children with cancer. The stability of rule 8.1b is critical in the case of supporting a wellbeing agenda with positive outcomes for New Zealand society and in ensuring New Zealand is the best place to bring up children.

We recommend Rule 8.1(b) of the Pharmaceutical Schedule is maintained as it is currently devised. To make any decisions that limit, change or eliminate rule 8.1(b) within the context of the current knowledge and the information provided would not be evidence-based and would risk decisions being made on faulty assumptions or incorrect use of data.

RDNZ believes that the best people to make decisions on treatments are parents and their medical professionals, Rule 8.1(b) allows for this. As such we recommend that should there be any changes to the rule ongoing engagement with senior clinicians is paramount in a co-design process.

Rare Disorders NZ believes rule 8.1b should be extended for all rare disorders, not just those that are rare cancers which would currently come under this rule. We believe and advocate for this to support an equitable wellbeing agenda with positive outcomes for New Zealand society and in ensuring New Zealand is the best place to bring up children.

Question 5
To what extent are good health outcomes for children with cancer in New Zealand dependent on making paediatric cancer treatments available through rule 8.1b?

Rule 8.1b plays an important role in the treatment of paediatric cancer patients in New Zealand to the extent that health outcomes for many in this patient population are dependent on access to novel treatments. Taking this option away or reducing in anyway will harm children through reduced outcomes for childhood cancers.

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RDNZ believes that the best people to make decisions on treatments are parents and their medical professionals, Rule 8.1(b) allows for this. As such we recommend that should there be any changes to the rule ongoing engagement with senior clinicians is paramount in a co-design process.

Rare Disorders NZ believes health outcomes for children overall would be improved if the rule was extended to other paediatric diseases or conditions, including children with rare disorders.

Question 6
Is timely access to paediatric cancer treatments more important than timely access to other medicines or for other populations? If so, why?

Patient populations which face similar economic characteristics to paediatric cancer, such as children with rare disorders, are likely to face similar issues and challenges with respect to medicines access. Many of these disorders carry high risk of morbidity and/or mortality so timely access to effective medicines is crucial to alleviate suffering and extend survival.

The potential gains made by early intervention with medicines for paediatric cancer and other rare childhood disorders are larger than many other patient populations, given the relatively young age of the patients and the expectation that treatment will result in extended survival with good quality of life.

Supporting timely access to medicines for paediatric cancer and other rare childhood disorders supports cost avoidance or reduced costs for other clinical and healthcare costs which is the right ethical path to take. Also supporting timely access to medicines for paediatric cancer and other rare childhood disorders supports children to grow into adults and become productive participants in New Zealand society.

Question 7
Is our understanding of how rule 8.1 operates in practice correct? What else should we know?

Timely access to modern medicines has the potential to improve health outcomes early and avoid costly hospital stays and medical interventions, such as surgery, further downstream. Conversely, restricted

access to effective medicines has the opposite effect of requiring ongoing costly medical interventions throughout the course of the cancer and disorder treatment.

The wider economic costs of not treating the disease early include loss of productive work or school hours, resulting in an increase demand for social welfare payments and services. There is also a social cost, which contributes to demand for mental health services, and is characterised by increased levels of stress, anxiety and depression among those affected. These demands on primary care and mental health services should not be under estimated, for example from our survey in 2022 with one in three people with a rare disorder are often unhappy and depressed and feeling they cannot overcome their problems. This is highly likely to be the same or worse for children with a paediatric cancer and their families.

Any changes to rule 8.1b which restricts access to paediatric cancer medicines by forcing new medicines to go through the regular Pharmac funding process has the potential to seriously impact health outcomes for childhood cancer patients.

In general, the drug development process is not conducive to producing new medicines to treat paediatric cancers. The requirement of regulatory authorities or drug funding agencies to require "gold-standard evidence" based on large, randomised clinical trials is a major obstruction for new paediatric cancer treatments. Some of the reasons for this include:

1. The clinical trials usually require a control arm where a significant number of patients are randomised to receive either standard of care and/or placebo;
2. To reach statistical significance the trials require large number of participants which is very difficult when it comes to paediatric cancers, due to the small and diverse patient population;
3. Many clinical trials exclude children, therefore the final marketing approval does not include children and doctors are required to prescribe the treatment "off-label";
4. The economics and logistics of these trials mean they are very costly to run, which is a negative incentive for the drug developers, usually biotech or pharmaceutical companies;
5. The small patient population, high drug development costs and limited patent lifespan combine to produce a high list price when the drug is finally approved;
6. A high list price is a disincentive for centralised drug funding agencies, such as Pharmac, to reimburse the drug.

Given these obstructive forces, many potentially effective new paediatric cancer medicines may struggle to progress successfully through the drug development process into late stage clinical trials.

If rule 8.1b were to change so that new medicines were required to undergo the normal Pharmac funding pathway, it is likely that access to these medicines will be greatly restricted, with a highly likely negative impact on children with cancers and their families, including clinical outcomes.

Question 8
How much increase in the use of rule 8.1b do you think will happen as a result of the growing range of new paediatric cancer treatments?

Without an increase in the Combined Pharmaceuticals Budget (CPB), resulting in a larger number of medicines funded by Pharmac, the use of rule 8.1b would be expected to rise as paediatric oncologists would have no other way of accessing the treatments their patients need. It is extremely important not to focus solely on the cos of drugs and the budget the financial costs of funding treatments under rule 8.1b will be greatly offset by lower medical costs, economic costs and social costs.

Question 9
Do you see the costs of paediatric cancer treatments accessed through rule 8.1b increasing significantly in the foreseeable future?

Rather than fear the costs of new treatments, Rare Disorders NZ believes that Pharmac should consider the revolutionary health benefits that many of these treatments can provide. A value-based funding approach, rather than a cost-based approach, should be employed to assess these benefits. This will also mean cost savings to not only the health system but every other social system in New Zealand.

Rare Disorders NZ believes rule 8.1b should be extended for all rare disorders, not just those that are rare cancers which would currently come under this rule. We believe and advocate for this to support an equitable wellbeing agenda with positive outcomes for New Zealand society and in ensuring New Zealand is the best place to bring up children.

Question 10
How could we assess what value paediatric cancer treatments provide against other medicines that could be funded with the same money?

Rare Disorders NZ believes Pharmac should be assessing value of all medicines not just on cost of the medicine (spend from medicines budget as part of Vote Health) versus health outcomes (as deemed within Vote Health) but using a values and wellbeing based approach, including all health, social determinates of health, social care, education, employment, family and societal impact.

When looking specifically at paediatric cancer, treatments are designed to be 'curative' as opposed to 'palliative'. Given the relatively young age of the patient, early intervention with new medicines can avoid a significant number of 'life years lost' due to premature death of the patient (in the absence of treatment). Delays in accessing new medicines can have the converse effect of many years of costly medical interventions in managing the patient's disease.

The effects of paediatric cancer are wide ranging and typically impact not only the child but their immediate family, wider whānau and community. Rare Disorders NZ firmly believes that any assessment of value of a paediatric cancer treatment should weigh up the direct cost of the medicine against the medical, economic and social costs incurred if the medicine is not funded.

Question 11
What should Pharmac take into account when considering equity issues with respect to rule 8.1b of the Pharmaceutical Schedule?

There is inequity in the current drug funding policy surrounding rule 8.1b because medicines for children with diseases or disorders other than cancer are not covered by the rule. Rare Disorders NZ believes that the health of all children in New Zealand should be prioritised and that extension of rule 8.1b to other paediatric diseases and rare disorders will help achieve this.

Any changes to rule 8.1b which result in reduced access to new medicines have the potential to increase inequities in the health system, as it exacerbates the situation of 'haves and have-nots' in regard to affordability of medicines. Families who are not in a position to self-fund their treatment, which may include Māori, Pacifica, those with a Rare Disorder, disability or groups with lower socio-economic status, will be disadvantaged. The current rule 8.1b eliminates this inequity for paediatric cancer patients only.

The paramount objective to take into account is to achieve the best health outcomes for *all* New Zealand children facing serious illness. Therefore, the key priority should be to expand rule 8.1b to lift all groups currently facing inequities in medicines access to the level of paediatric cancer patients enjoy under this rule.

Question 12
Do you consider rule 8.1b to be inequitable from the perspective of other children or those with rare disorders? Why?

Yes we consider rule 8.1b to be inequitable from the perspective of other children and those with rare disorders. Children with rare disorders and other chronic conditions are currently at an access and treatment disadvantage to paediatric cancer patients in terms of access to new medicines because rule 8.1b only applies to paediatric cancer medicines. Rare Disorders NZ would like to see this inequity removed by expanding the provisions of rule 8.1b to include all rare disorders and chronic conditions. We believe New Zealand should be lifting up and being inclusive to reach equity not reducing access and funding to meet equity.

Question 13
To what extent do the current policy settings, including rule 8.1b, contribute to the health outcomes achieved for tamariki Māori and Pacific children with cancer?

The current rule 8.1b, removes a vital layer of inequity in that the financial barrier of access to paediatric cancer medicines is removed. Without rule 8.1b, access to some medicines would be restricted to those that can afford them. Families in the lower socio-economic sector, of which Māori, Pacifica, the disabled and those with a rare disorder are over-represented in this lower socio economic group, will face a major barrier to access. This in turn leads to poorer outcomes in these groups, this inequity of outcome can be prevented through equity policy settings. Taking away rule 8.1b or reducing it in any way will create inequity. We believe New Zealand should be lifting up and being inclusive to reach equity not reducing access and funding to meet equity.

Question 14
Do you consider rule 8.1b to be inequitable from the perspective of adolescent and young adults with cancer? Why?

Yes, adolescent and young adults with cancer face many of the same challenges and issues as children with rare disorders and chronic conditions yet they lack effective treatments in New Zealand that would be available to them in other OECD countries. We believe New Zealand should be lifting up and being inclusive to reach equity not reducing access and funding to meet equity.

Question 15
How might we address equity and fairness concerns related to paediatric cancer medicines through rule 8.1b and access to medicines for other groups?

Extend the criteria of 8.1b to include rare disorders and chronic illness. Children and adolescents are one of the most vulnerable groups in our society and Rare Disorders NZ firmly believes that the health of all children should be prioritised.

Rare disorders either life-limiting or cause of serious morbidity can lead to poor quality of life. The effects of these disorders on the patient and whānau are similar to those for paediatric cancers and deserve the

same standard of care and access to modern medicine as those who have cancer. We believe New Zealand should be lifting up and being inclusive to reach equity not reducing access and funding to meet equity.

Question 16
Is there anything else we need to know to inform the review? If so, please add your information or thoughts here.

Early access to modern, targeted medicines has the potential to extend overall survival and preserve high quality of life. The cost of funding this early access can be offset against the cost of non-pharmaceutical medical interventions, and the associated economic and social costs, incurred throughout the disease trajectory by both the patient and their wider whānau.

RDNZ believes the broader issue vis-à-vis technologies like CAR T-cell therapy and how it will be funded is a distraction from the issue at hand and as such should be excluded from any debate around rule 8.1(b).

Even though Trikafta and Spinraza have been funded, there are still medicines that need to be funded for those with a rare disorders in New Zealand including but limited to Myozome for Pompe, medicines that treat Fabry disease, the unfunded steroid for Duchenne.

The economic and humanistic argument which supports the principles behind rule 8.1b can be equally applied to other childhood diseases. Therefore, Rare Disorders NZ supports the expansion of rule 8.1b to include all children with serious illnesses, including those with rare disorders and other life-threatening illnesses where there is a medicine available – currently funded or not. Not only life threatening illnesses but also conditions where early treatment would prevent decline and ill health later in life. Early treatment is essential for long term health and development of children and their future well-being.

Leadership is now needed to extend the rule 8.1b to include medicine for children with rare disorders and chronic illness in New Zealand.

ENDS

Rare Disorders NZ

20 March 2023