Summary of submissions to the review of rule 8.1b of the Pharmaceutical Schedule

March 2024

PHARMAC
TE PĀTAKA WHAIORANGA

Acknowledgement

Pharmac would like to acknowledge the significant emotional labour and time it took for submitters to respond to this consultation. In our discussion document inviting submissions we said that we appreciated that cancer and its treatment can have profound health, emotional, social, educational, and economic impacts on children, their family and whānau. We said that the prospect of possible change may be unsettling for many people.

These vast impacts were apparent in the detailed and personal submissions we received from family and whānau and from the clinicians and NGOs whose lives are dedicated to treating those diagnosed with a paediatric cancer and supporting those around them. The submissions outlined in detail the day-to-day and lifelong impact of having a child diagnosed with cancer, including the horror and devastation wrought when a child dies.

All families and whānau who submitted told us about their own child's story – from fighting for diagnosis to years of treatment, to potentially life-long impacts of that life-saving treatment. Some told us about their child's death, and others told us of the constant fear of relapse. Alongside this, families and whānau put many hours of research into their submissions, delving into scientific papers and journal articles – a skill many had picked up when their child was diagnosed with cancer.

We would also like to acknowledge that there were countless families and whānau who may be affected by this review who did not submit for many reasons, including that the time and emotional labour required was not something they currently had the capacity for. Families and whānau who submitted specifically spoke to this fact ("I know of a lot of families out there who are not engaging in it because it's too hard"). In line with the unity between families and whānau living through a child's cancer diagnosis and treatment, some families and whānau shared the experiences and stories of others, with their permission, to ensure their voices were recorded. We thank you for this.

As a content note, in order to authentically capture the voices of families and whānau we have used direct quotes from submissions that talk about the sickness and death of children.

Privacy

As part of this consultation process, we heard the personal stories of families and whānau of those diagnosed with a paediatric cancer. We have taken care to preserve their privacy when using their voices in this document. Names, genders, and other identifying details (such as the type of cancer) have been removed.

The illustrative quotes used in this document may have been lightly edited for clarity – any additions that are not directly from the individual are indicated by [square brackets].

If you have concerns with how we have portrayed the stories of families and whānau, please contact us at consult@pharmac.govt.nz. Additionally, if you were involved in this consultation and would like a copy of the personal information we hold about you, or to correct any information that is wrong, please make a Privacy Act request[2] through privacy@pharmac.govt.nz.

We hope that we have treated the stories of family and whānau with care.

2 Pharmac's guide to making Privacy Act requests can be found here - https://pharmac.govt.nz/news-and-resources/official-information-act/making-a-privacy-act-request/

Purpose

This paper is a summary of the submissions received in response to the discussion document published in November 2022. As a summary of submissions, it is reflection of a synthesis of submitters views and opinions. It therefore may not fully reflect the views in any one submission, and be contradictory in places, as submitters did not have a unified view on many of the issues raised in the discussion document. This paper is not Pharmac's position paper on the future of rule 8.1b.

Executive Summary

Te Pātaka Whaioranga – Pharmac is reviewing rule 8.1b of the Pharmaceutical Schedule. In essence, rule 8.1b provides clinicians with the full decision-making authority to prescribe medicines that are not publicly funded to children diagnosed with cancer and who are treated in a paediatric setting.

Pharmac is reviewing rule 8.1b for two reasons. First, questions have been asked about whether it is equitable for paediatric cancer medicines to be treated differently to medicines for other childhood diseases and conditions. Second, Pharmac has limited ability to monitor the use of rule 8.1b and manage any cost growth.

Pharmac would like to reiterate that whatever the results of this review, all treatments currently accessed through rule 8.1b will continue to be available for existing and new patients.

To complete the first stage of the review, Pharmac published a discussion document in November 2022 and invited submissions to better understand the difference that rule 8.1b makes to the lives of of children with cancer, their family and whānau, and the people working hard to support them. The discussion document presented sixteen questions (attached as an appendix to this document) under five main themes, which were:

a. how well do we understand child cancer and the system of care?
b. how effective is rule 8.1b in terms of achieving the best health outcomes?
c. does the current policy support efficient and sustainable use of available resources?
d. does the current policy support equity?
e. other information or thoughts?

Pharmac received 86 submissions. Submitters were made up of families and whānau of children who have been treated for cancer, paediatric oncologists and paediatric haematologists, clinicians who treat adolescent and young adults with cancer, NGOs that support and advocate for children and young people with cancer and other rare diseases, pharmaceutical companies and concerned individuals.

The submissions engaged closely with the questions and provided a wealth of information and stories that will strongly contribute to this review.

To clarify, rule 8.1b is a subsidy for any pharmaceutical for use within a paediatric oncology/haematology service for the treatment of cancer. In past documents the phrase ‘children with cancer’ and ‘paediatric cancers’ have been used interchangeably. There is an important distinction between the two. Children with cancer speaks to the age of the person while paediatric cancers speak to the types of cancer a person, of any age, maybe be diagnosed with.

In Aotearoa New Zealand, nearly all people up to 16 years of age are treated in paediatric cancer services, therefore eligible for access to rule 8.1b, however, adolescents and young adults with a paediatric cancer treated within an adult setting and are not eligible.

There were ten overarching themes from submitters about rule 8.1b and its contribution to outcomes for those diagnosed with a paediatric cancer in Aotearoa New Zealand. These were:

a. rule 8.1b should be maintained and/or extended to other groups
b. children should be prioritised over adults in health spending
c. rule 8.1b is why there are equitable outcomes for tamariki Māori with cancer
d. rule 8.1b is why Aotearoa New Zealand has comparable outcomes with other countries for paediatric cancer, and equity across location ethnicity, and socio-economic status
e. rule 8.1b funds medicines that are the “standard of care” in other countries
f. rule 8.1b is the reason those diagnosed with a paediatric cancer in Aotearoa New Zealand can access clinical trials
g. paediatric cancer is different to other rare diseases and to adult cancer
h. if rule 8.1b was removed and / or changes were made to require an application process for access to paediatric cancer medicines, clinicians would be taken away from patient care and families and whānau would experience even greater levels of stress
i. if paediatric cancer medicines had to follow the standard Pharmac process, a two-tiered system would be created, where some children will be able to be treated through private funding and some will not
j. adolescents and young adults can also be diagnosed with a paediatric cancer. They require equitable access to paediatric cancer treatment under rule 8.1b to ensure they can participate in clinical trials and access standard of care treatment regardless of where they are treated.

Who submissions were from

We received a total of 86 submissions through an online form, via email and by video submission. The table below outlines the number of submissions by type of submitter.

Type of submitter	Number of submissions
Whānau Māori of a tamaiti who had, or has, cancer (one whānau submitted in writing and a video, this has been counted as one submission)	**11**
Pacific family of a child who had, or has, cancer	**1**
Families of a child who had, or has, cancer	**27** Including seven video submissions
Individuals who had been treated for cancer as a child or an AYA (including a group submission)	**3** One of these people was Māori
Clinicians and clinician groups	**10** Three of these were group submissions
Non-government organisations (one was part of a consensus submission and counted as one submission)	**18**
Pharmaceutical companies	**4**
Concerned individuals	**12** One of these people was Māori
TOTAL	**86**

In relation to the **family and whānau submissions**, 32 of these were written, seven were in video form and one whānau Māori submitted a written submission accompanied by a video submission (this is counted as one submission). Most family and whānau submissions were from parents, but also featured siblings, aunts, uncles, grandparents and godparents.

Some submissions from family and whānau also talked to the experiences of other families and whānau who had neither the emotional energy nor time to make a submission of their own. Additionally, in the preparation of their submission, the Child Cancer Foundation consulted extensively with their parent community and submitted six video submissions from families and whānau with their submission (these were treated as individual submissions from whānau).

Many **submissions were received from clinicians**. These included the National Child Cancer Network, a consensus statement from 14 paediatric oncologists and paediatric haematologists, and an endorsement of the AYA Cancer Network Aotearoa submission by several clinicians involved in the treatment of AYAs with cancer. Submissions were also received from clinicians within the Children's Oncology Group and the Medicines Utilisation Service in the Department of Pharmacology, Te Whatu Ora.

In relation to **submissions from individuals who had been treated for cancer as a child**, two were from individuals and one was a group submission from the AYA Cancer Consumer Advisory Group, a group that represents a diverse group of AYA across Aotearoa who have had a diagnosis of cancer. This submission was submitted alongside the submission from the AYA Cancer Network, but was treated as a separate submission.

We recieved a number of **submissions from non-government agencies** (NGOs) including those that support and advocate for people with cancer and rare disorders, as well as those committed to clinical research.

The four **pharmaceutical companies** were AbbVie, Janssen (the Pharmaceutical Companies of Johnson & Johnson, hereafter referred to as Johnson & Johnson), Merck Sharp & Dohme, and Biogen.

In relation to the **submissions from concerned individuals**, they may have had specific interests in rule 8.1b and paediatric cancer care, however they are in this category as that interest was not stated. One of these 'concerned individuals' was Māori.

Terms used in this analysis

The following terms used throughout the report have the following meanings:

- "most" means 50% or more submitters (50% ≤ x)
- "many" means between 30% and 50% submitters (30% ≤ x < 50%)
- "some" means between 12% and 30% submitters (12% ≤ x < 30%)
- "a few" means less than 12% submitters (x < 12%)

Part One: overall themes

This section outlines the overall themes of the submissions. To be included as an overall theme the point had to be made by more than 15 submissions.

1.1. Rule 8.1b should be maintained, and/or extended to other groups

The overwhelming theme from submitters was that rule 8.1b of the Pharmaceutical Schedule must be retained, or if changes were made, those changes should be to extend the rule to include other groups, such as:

- children with rare disorders
- AYA aged 15 to 24 with paediatric type cancers regardless of treatment setting
- AYA with any cancer.

Submissions from whānau Māori all said, in some way, that rule 8.1b should be retained, and the submission from a Pacific family said, *"I am writing this submission in support of rule 8.1b or an alternative to it that continues to allow the same funding options or more for children in New Zealand with Cancer."*

> ❝ The whole children's cancer treatment system of care relies entirely on the provisions contained within rule 8.1b ❞
>
> *National Child Cancer Network*

> ❝ Removing rule 8.1b of the Pharmaceutical Schedule is UNACCEPTABLE ❞
>
> *Emphasis added by submitter, an individual treated for cancer as a child*

> ❝ [The review] makes me feel sick, it makes me feel sad, it makes me feel mad❞
>
> *Family submission, a close family member of a child who died of cancer*

> ❝ For a significant minority five-year survival is entirely dependent on access through 8.1b ❞
>
> *Consensus submission from paediatric oncologists and paediatric haematologists in Aotearoa New Zealand*

These quotes illustrate that submitters were scared that the main option under consideration was removal of rule 8.1b, will all medicines needing to go through standard Pharmac processes.

The second part of this theme was that many submitters said the answer to perceived inequity between paediatric cancer and other childhood diseases was to "level up not down" or "equity up not equity down", and therefore extend rule 8.1b to other groups. Many submissions from whānau Māori also made this point. It is relevant to note that there were submitters that supported rule 8.1b only applying to paediatric cancer because of it being different to other paediatric conditions.

There was concern among submitters that medicines would be taken away from those diagnosed with a paediatric cancer to "solve" the equity issue. Submitters countered that the appropriate solution was to extend rule 8.1b to other children.

One submission from parents whose child is currently being treated for cancer and are waiting for better medicines to be developed for their child to have access to said that *"we agree that the current rule may be inequitable when taking other illnesses into consideration. However it seems perverse – even cruel – to suggest that the pathway to achieving health equity is to take services away from those who need them. The only reasonable approach must be to increase services to people who currently do not receive them".*

> ❝ Equity Up is the solution… Responding with Equity Down and reducing access will increase unfair, inequitable access for children with cancer and their whānau. The rule creates fit for purpose care – [it should only be taken away if] the goal is to expand inequitable health ❞
>
> *Submission from a Māori whānau*

> ❝ But the answer is not to remove rule 8.1b… Rather New Zealand should extend… to fund treatments for other conditions that affect children ❞
>
> *Family submission*

1.2. Children should be prioritised over adults in health spending

Along with whānau Māori submitters, the Child Cancer Foundation discussed the te ao Māori concept of he taonga te tamaiti, children are our treasures. A submission from a Māori whānau who had lost their tamaiti to cancer said that "tamariki are at the centre of whānau, hapū and iwi structures" and spoke about the Crown's obligation to enact policy which upholds and protects the interests of tamariki. Other submitters spoke to the same concept. This was often in reference to the status of children as a vulnerable or special population protected by international obligations and general societal expectations about the treatment of children:

> ❝ ...tamariki and rangatahi are 'in and of themselves' a special population as recognised by NZ ratifications of international law and broader national/ societal values ❞
>
> *Medicines New Zealand*

> ❝ You cannot put a dollar value on a child's life...we must always do the best for children ❞
>
> *Family submission*

> ❝ The numbers may be small...but they are not insignificant, they are our tamariki ❞
>
> *Parent of a child who was treated for cancer*

1.3. Rule 8.1b is why there are equitable outcomes for tamariki Māori with cancer

Where submitters talked about equitable outcomes between tamariki Māori and non-Māori, they were clear that the reason for equitable outcomes between those groups of children was the existence of rule 8.1b/a mechanism for all children having access to the same treatment. This was a key focus of submissions from whānau Māori.

Submitters outlined the obligations of Pharmac and the broader health and disability system under the Pae Ora (Healthy Futures) Act 2022 and the obligations of the Crown under Te Tiriti o Waitangi in this discussion, submitting that Pharmac had not properly considered its obligations under Te Tiriti o Waitangi. The Child Cancer Foundation submitted that they had *"already been asked if removal of rule 8.1b could be the subject of a Waitangi Tribunal determination".*

Many submitters commented on the fact that paediatric cancer outcomes were unusual in their achievement of equitable outcomes between tamariki Māori and non-Māori children aged 0-14 and queried why Aotearoa New Zealand would want to take the risk of losing that ground. This was a common thread made across all different types of submissions. However, it was most prevalent in submissions from clinicians.

While most submitters that discussed equity between tamariki Māori and non-Māori children spoke about it in the terms above, the Child Cancer Foundation submitted that the survival rates outlined in the discussion document, *"disguises inequalities that exist for Māori, who between 2010 and 2019 had a 5-year survival rate of 81%, 7% below non-Māori"*. The consensus submission from paediatric oncologists and paediatric haematologists noted that work was on-going to understand inequities in survival outcomes between Māori aged 10-14 with cancer, and non-Māori of the same age with cancer.

> ❝ In a health system where inequity between Māori and non-Māori exists everywhere, we take this opportunity to advise Pharmac that complicating or weakening access to paediatric cancer treatments...will...result in a two-tier system for cancer treatments in paediatrics...likely to disproportionately affect Māori whānau and Pacific peoples ❞
>
> *Consensus submission from paediatric oncologists and paediatric haematologists in Aotearoa New Zealand*

1.4. Rule 8.1b is why Aotearoa New Zealand has comparable outcomes with other countries for paediatric cancer, and equity across location, ethnicity and socio-economic status

Many submitters raised that paediatric cancer survival rates were comparable to other countries because children have access to the same medicines. Submitters said this was not the case for other diseases, including adult cancer. They therefore submitted that the difference could partly be explained by lack of access to medicines. The countries frequently referred to were Australia, Canada, the United Kingdom, and the United States. This submission was made by all types of submitters including whānau Māori, but particularly by clinicians and NGOs.

> ❝ We state categorically that continuation of our ability to benchmark survival is dependent on continued access to cancer pharmaceuticals... ❞
>
> *Consensus submission from paediatric oncologists and paediatric haematologists in Aotearoa New Zealand*

> ❝ [Rule 8.1b] is likely to be the determinative factor in the comparability of outcomes with our peer nations ❞
>
> *Medicines New Zealand*

As well as discussion about equitable survival outcomes with other counties, submitters spoke about equitable outcomes within paediatric cancer. They stated that because of the system of care for paediatric cancer, of which they said rule 8.1b was an integral part, there was no difference seen between children from different geographic locations across Aotearoa New Zealand, different ethnicities and across different socio-economic statuses.[3]

3 The inequitable survival outcomes between rangatahi Māori and non-Māori AYA are addressed by some submitters and discussed later in this report.

> ❝ New Zealand can be justifiably proud of the paediatric cancer survival outcomes. It highlights world class survival rates for all children, irrespective of age, ethnicity, or where they live. These outcomes are significantly dissimilar to patients treated under the adult regimen – for key (and hopefully obvious) reasons ❞
>
> *Leukaemia and Blood Cancer New Zealand*

1.5. Rule 8.1b funds *"standard of care"* medicines

A high number of submissions from clinicians said that rule 8.1b funds '*standard of care*' medicines. Clinicians said that the medicines the rule is funding are not 'novel' or 'niche' but are accepted as standard treatment for paediatric cancer. Generally, these submitters said that most of the medicines funded through rule 8.1b were publicly funded in comparable countries (most frequently cited was Australia). This submission of clinicians was supported by submissions by NGOs, families and whānau Māori and concerned individuals that one of the reasons rule 8.1b was needed was Aotearoa New Zealand's low Combined Pharmaceutical Budget (CPB).

In support of the medicines accessed by the rule being *"standard of care"* pharmaceuticals, the Medicines Utilisation Service in Waitaha Canterbury (Waitaha Canterbury), the consensus submission of the paediatric oncologists and paediatric haematologists and the Australia and New Zealand Children's Haematology/Oncology Group (ANZCHOG) all conducted reviews of the use of rule 8.1b.

The consensus submission performed a descriptive data analysis of all paediatric cancer treatment (PCT) notifications made between 2019 and October 2022 and concluded that 170 out of 242 (or 70%) were for *"standard of care"* indications and said they felt comfortable justifying every use of rule 8.1b.

The ANZCHOG Board reviewed the list of agents prescribed using rule 8.1b between June 2018 and September 2022 and *"found none that would be considered non-standard in the Australian children's cancer context"*.

Waitaha Canterbury submitted that between June 2018 and September 2022 there were 57 children who received medicines through rule 8.1b in Canterbury, *"of those, 39 children [68%] who received medicines via this rule were following established protocols for treatment of childhood cancers"*.

As well as submitting that most of the medicines accessed through rule 8.1b were *"standard of care"*, the consensus submission from paediatric oncologists and paediatric haematologists said that some medicines accessed through rule 8.1b were on the Pharmaceutical Schedule. They said that they accessed them through rule 8.1b because these medicines were on the Pharmaceutical Schedule for other indications. This is part of a broader point made by other submitters that it is rare for medicines to be developed specifically to treat paediatric cancer, and therefore medicines developed for other purposes are used to treat paediatric cancer.

1.6. Rule 8.1b is the reason those diagnosed with a paediatric cancer in Aotearoa New Zealand can access clinical trials

For those who submitted about clinical trials, most agreed that rule 8.1b was *"crucial"* to children and AYA with cancer to access cancer clinical trials in Aotearoa New Zealand. Some whānau Māori made this submission. The cruciality was based on submissions from clinicians and NGOs stating that participation in a clinical trial is not only dependent on being able to access the trial drug (which is usually supplied free of charge by those conducting the trial) but also on having access to *"first-line cancer treatment"*, some of which is accessed through rule 8.1b. The Child Cancer Foundation[4] submitted that without this access *"children [would be] ineligible for enrolment in clinical trials"*, and this was repeated by other submissions from clinicians and NGOs.

Submitters agreed that paediatric cancer clinical trials in Aotearoa New Zealand was highly sensitive to changes. Given that 20 to 40 percent of paediatric cancer patients are enrolled in clinical trials, a change as significant as the removal of 8.1b would be hugely impactful.

Submitters said that removal of rule 8.1b would not only reduce the likelihood of survival for those diagnosed with a paediatric cancer but would also impact on Aotearoa New Zealand's ability to retain skilled clinicians and access the broader benefits of clinical trial participation (including ready access to international expertise).

4 Note that the Child Cancer Foundation submission was supported by CanTeen Aotearoa, Cure Kids and Ronald McDonald House Charities (RMHC) New Zealand.

1.7. Paediatric cancer is different to other rare diseases and to adult cancer

In reference to the reasons for the existence of rule 8.1b, some submitters spoke about paediatric cancers in children and AYA being clinically and biologically different to adult cancer and other rare diseases. One submission from whānau Māori submitted that paediatric cancer was different to adult cancers. The differences between paediatric cancer and adult cancer, and other rare diseases, that were drawn out by submitters included:

a. paediatric cancer tends to be more aggressive and requires fast access to treatment
b. paediatric cancer is the leading cause of death among children in New Zealand, outside of unintentional injury
c. paediatric cancers are biologically different to adult cancers
d. treatment given for paediatric cancer aims to be curative and for a limited time, which is not the same for other rare diseases. It should be noted here that when making this point some submitters expressed a level of discomfort with trading the curative nature of cancer off against life-long conditions.

> ❝ The consequence of not having rule 8.1b without a fit-for-purpose revised equivalent pathway, would mean countless hours of burdensome applications, with delays in approvals beyond what may be clinically safe, the risk of inconsistent approvals and the potential reliance on decision makers without sufficient paediatric oncology expertise ❞
>
> *Child Cancer Foundation*

1.8. If rule 8.1b was removed or if changes were made to require an application process for access to paediatric cancer medicines, clinicians would be taken away from patient care and families and whānau would experience even greater levels of stress.

Many submitters were concerned about additional stress being placed on families and whānau, and clinicians being taken away from patient care, if rule 8.1b was removed or changed.

Submitters were concerned about the amount of time it would take if clinicians had to make funding applications. Whānau Māori submitted frequently on this point. Families and whānau who had been through medicines application processes such as the Named Patient Pharmaceutical Assessment (NPPA), submitted that the process was highly stressful and did not want them to be required more often. The consensus submission of the paediatric oncologists and paediatric haematologists outlined the *considerable risks* of the removal or change of rule 8.1b. They said that:

a. a similar gap to adults in access to medicines would arise
b. there would be a long lead-in time to wait for *"novel but inexpensive therapies"*
c. the NPPA was a *"poor mechanism"* in the small paediatric cancer network
d. Pharmac mechanisms had not delivered fair or equitable outcomes for Māori or Pacific patients, where rule 8.1b has.

1.9. If paediatric cancer medicines had to follow the standard Pharmac process a two-tiered system would be created, where some children will be able to be treated through private funding and some will not.

Submissions related to this point expressed a high level of fear. They described this two-tiered system as *"the haves and the have nots"* where families with the financial, educational, and emotional resources would be able to access medicines their children needed compared to those who would not. Submitters said this would mean more children would die. The tone of submissions on these points are captured by the following quote from a parent whose child was treated for cancer:

> ❝ There is nothing more levelling than being on the children's cancer ward. Every room holds its own heart-breaking story of a child's struggle with cancer…everyone is equal and united in the task of nursing their child through the horrificness of a cancer diagnosis. Currently, every single child on that cancer ward is getting access to the medicines they need. I can't imagine how it would be if there was an inequality of access to medication… two families in the same cancer ward, one has a child on a better regime because they can pay for it… could you imagine the despair and heartbreak of being that family that couldn't afford the best and kindest medication for their child? ❞
>
> *Family submission*

1.10. Adolescents and young adults (AYA) can also be diagnosed with a paediatric cancer. AYAs require equitable access to paediatric cancer treatment under rule 8.1b to ensure they can participate in clinical trials and access standard of care treatment, regardless of where they are treated.

Submissions received from clinicians treating AYA with cancer highlighted the importance to note that AYA can be diagnosed with a paediatric-type cancer. Paediatric cancers occur most commonly but not exclusively in the paediatric population (commonly defined as 0-14 years of age).

Outcomes for children with cancer continue to improve irrespective of age, ethnicity or where they live. Unfortunately gains in survival outcomes over the past 20 years have not been as marked in the AYA cancer population compared to the paediatric population. Greater access to paediatric clinical trials and paediatric treatment protocols for AYAs in recent years has certainly played a part. But there is still much work to be done to improve Aotearoa New Zealand's survival rates and to address health inequities for those aged 15 years and over who are diagnosed with a paediatric cancer.

Part Two: Detailed summary of submissions

Detailed feedback from whānau Māori

Pharmac received 11 submissions from whānau Māori. Submissions spoke about the operation and impact of rule 8.1b on equity for tamariki and rangatahi Māori and about Pharmac and the Crown's obligations under Te Tiriti o Waitangi.

Given those obligations, Pharmac considers it important to have a dedicated analysis in this report of what whānau Māori told us in response to the discussion document, as well as outline the submissions about tamariki, rangatahi and whānau Māori in the general detailed feedback you can find below.

2.1. How well does Pharmac understand child cancer and the system of care?

In response to questions about Pharmac's understanding of child cancer and the system of care, three submissions from whānau Māori talked about children's access to clinical trials, and the dependence of clinical trial access on rule 8.1b.

One parent said it was their understanding that if medicines could not be accessed through rule 8.1b *"...many children will not get access to clinical trials"*. The two others said that clinical trials were very important for advancements in all cancer treatments, with one adding that without the ability to participate in clinical trials in paediatric cancer settings they expected Aotearoa New Zealand would *"lose specialist doctors and nurses"*.

2.2. How effective is rule 8.1b in terms of achieving the best health outcomes?

In response to questions about how effective rule 8.1b was in achieving the best health outcomes, the submissions from whānau Māori were unanimous in their statements that rule 8.1b led to good health outcomes. One whānau said *"rule 8.1b protects tamariki and their whānau through equitable access to all medication"* and that *"it is really hard to believe we are facing a review about limiting funding and changing rule 8.1b"*. Another submitted that *"your statistics show child cancer outcomes being largely favourable, why would you consider any backwards step to this?"*

Submissions from whānau Māori expressed relief that rule 8.1b meant that the best health outcomes were available via access to medicines in Aotearoa New Zealand, with one submission talking about the impact of their child being able to be treated close to home.

> "[They were] able to be treated close to [their] home, where [they] felt safe... and surrounded by [their] loving family/whānau. When children are vulnerable and acutely unwell, this is of utmost importance. As parents, we remain thankful that we did not have to suffer the additional emotional burdens and financial hardship of being forced to travel overseas to access the best treatment for our child because of Rule 8.1b"
>
> *Child Cancer Foundation*

Two submissions talked about the importance of timely access to medicines for tamariki with cancer, saying that for their tamariki immediate treatment was needed. Another made a general statement about the importance of timely access to medicines:

> "...surprised and pleased [about] how quick treatment was there and available"
>
> *Child Cancer Foundation*
>
> "immediate start of treatment means within a week...prompt access must remain protected...every time we faced a poor treatment response or relapse, our paediatric oncologist was able to respond quickly and in line with overseas developments...rule 8.1b made this possible"
>
> "Access to medication immediately can potentially save a child's life"
>
> *Submissions from whānau Māori*

Submissions from whānau Māori said rule 8.1b meant that the system did not rely on whānau having to spend their time advocating for their tamariki and fundraising to pay for the costs of treatment. Whānau talked about the psychological and emotional impact of a child cancer diagnosis on parents, siblings and other whānau members, describing the trauma of the situation, and feelings of helplessness:

> "Child cancer has so many stressors, being able to access the right drugs shouldn't be one of them"
>
> "Not having rule 8.1b would cause immeasurable amounts of stress, pain and hurt during an already turbulent time"
>
> "With all that we have to face, not having to worry about being able to access the best treatment and medicines is one less thing to have to fight for"
>
> *Submissions from whānau Māori*

One submission outlined that a system that relied on advocacy could lead to poorer outcomes for Māori. They referenced research published in 2020 which reviewed two decades of qualitative research about Māori experiences of the health system, outlining that there were three areas which estranged Māori patients and their whānau within the healthcare system. They submitted that the research said an important barrier was that Māori patients and whānau had difficulty accessing resources and support because they were *"hesitant to self-initiate help"*. The authors of the review said that *"this self-silencing in order to avoid pressuring staff led to poorer health outcomes for Māori patients"*.

The whānau in this instance linked this to their own experience, saying that their child's Māori grandparent would often comment on their *"outspoken, adversarial, and pushy"* approach to accessing treatment. They said they were proud, however would not have the same confidence and *"wouldn't know where to start"*. The submission posed the question *"How many whānau may be more disadvantaged if they had to advocate for their child to get access to treatments currently afforded automatically under 8.1b?"*

One submission from a parent whose child had died of cancer said that rule 8.1b made a key difference to the experience of Māori and Pacific families:

> "For Māori and Pacific families, the rule makes a key difference to their experience. Time is the most precious resource for families at this time, and this preserves it and makes sure they don't have to spend time advocating and fundraising"
>
> *Submission from a whānau Māori whose child had died of cancer*

Submitters feared that the removal of rule 8.1b would *"increase the burden on parents and oncologists"*, particularly if funding applications needed to be made by clinicians. One whānau submitted that if a child relapsed, which they understood was often a situation where rule 8.1b was used,[5] there would be limited options *"but to wait for death with no sense of hope or control"*. Another whānau commented that their child's treatment was *"seamless"*, and that *"everything [their child] needed was right there"*.

A final submission point relating to rule 8.1b achieving the best health outcomes from whānau Māori was the *"hope"* that the existence of the rule provided, and how important that was:

> ❝ knowing they have access to medication gives [those diagnosed with a paediatric cancer] hope ❞
>
> ❝ the only hope you have is medication ❞
>
> ❝ hope lies in possibility ❞
>
> ❝ [On removing rule 8.1b] "you're taking away the fuel that burns inside to save your child ❞
>
> *Submissions from whānau Māori*

5 This is a submission that was made by several types of submitter, including clinicians.

2.3. Does the current policy support efficient and sustainable use of available resources?

The central theme of submissions from whānau Māori about whether rule 8.1b supported the efficient and sustainable use of available resources, was that because the rule supported the achievement of equitable outcomes between tamariki Māori and non-Māori children, it was an efficient use of available resources.

One submitter advised Pharmac to *"think broadly"* about equity, given Māori and Pacific peoples were not well looked after in the health system. Another commented that *"in comparison to current government spending, the costs are small"*.

In line with that theme, submitters talked about societal and ethical obligations towards children, *"Children are our taonga…our precious taonga,"* and about the obligations of the Crown to protect and uphold the interests of tamariki Māori. One submission said that tamariki are at the centre of whānau, hapū and iwi structures and referenced to section 7(d)(i) of the Pae Ora (Healthy Futures) Act 2022 which states that the health sector should resource services *"to meet the needs and aspirations of iwi, hapū and whānau"*.

On the theme of investing in tamariki, submitters said:

> ❝ …specialised treatments will always be expensive, but denying children the best treatments is unethical ❞
>
> ❝ Even if it's only one child that needs this rule, it's worth it ❞
>
> ❝ We need to invest in children, they are our future ❞
>
> ❝ If [my child who died] was here with me [they] would say we are worth funding and [they] would say adults please fight for us. Every child would say that ❞
>
> ❝ …no child should be denied access to the best possible treatment this country has to offer ❞
>
> *Submissions from whānau Māori*

One submission quoted the following whakataukī: *"Ehara taku toa i te toa takitahi, engari taku toa he toa takimano"* meaning *"my strength is not mine alone, but the strength of many"*. They said that the potential removal of rule 8.1b would place *"children's protectors",* their parents and oncologists, in *"helpless and powerless positions"*.

2.4. Does the current policy support equity?

One submission from whānau Māori talked about the *"battle to get access to quality care"*. They said that prior to their child's diagnosis with cancer they had not had much interaction with the health system and believe that the issues they faced with *"concerns not being taken seriously"* and *"being talked down to"*, as well as their child's treatment injury *"in some way is because of the institutional racism that we faced"*.

Another submission also made this point, saying that there was a delay to their child's diagnosis because the genetic markers of the disease did not match what clinicians were expecting because they were Māori.

Submissions from whānau Māori agreed with each other that the existence of rule 8.1b supported equity between tamariki Māori and non-Māori children, and supported equity between other groups. Two submissions that highlighted this said:

> ❝ Rule 8.1b means that despite our challenges, I know that we will always be offered the best treatment options, with the best medications to treat [our child's] cancer. We have had lots of battles with the system and I know that Māori are always worse off when there is a reduction in spending. Without equal access for all paediatric cancer patients, it will cause greater inequity ❞
>
> ❝ I would reiterate how equitable I found the paediatric cancer treatment in NZ. As far as I could tell there was little difference between the levels of care given to families from all backgrounds ❞
>
> *Submissions from whānau Māori*

In relation to equity between tamariki with cancer and tamariki with other rare diseases, most submitters who talked to this point supported an "equity up" solution of ensuring those tamariki also had access to the medicines they needed, including one submission that expressly said the rule should be expanded to include adolescents. One whānau commented that *"the review is in the interests of fairness and equity…this should not mean we make everything the same"*.

The same submission said that the approach of removing rule 8.1b to achieve equity of access between tamariki with cancer and tamariki with other diseases was *"a harmful approach that goes against government initiatives, legal, legislative and Te Tiriti o Waitangi principles"*. Another submission supported this point saying, *"comparing paediatric cancers with other illness will never have a satisfactory outcome – the question should be about how they could achieve equity with other illnesses, rather than removing funding from paediatric cancer"*.

Two further submissions from whānau Māori said that rule 8.1b aligns with the Crown's obligations to Te Tiriti o Waitangi. One whānau said that in their view *"8.1b aligns with Te Tiriti"* and asked *"What Pharmac would do to honour Te Tiriti (specifically Article 2)"* if the rule was changed. Another Māori parent said they would like to know *"how Pharmac's commitments to being a good treaty partner is impacted by this [potential] decision"*.

Finally, and linked to the submissions described above about removal of rule 8.1b leading to whānau needing to spend time fundraising and advocating, submitters talked about the barriers that would be in place if there was a possibility of whānau having to self-fund medicines:

> ❝ How could a parent face a situation where there is a treatment available, but you have barriers of having to pay, or others, and can't get it? How do you live with the fact that your child did not get a chance to have their life saved? ❞
>
> *Submissions from whānau Māori*

Detailed feedback from all submitters

2.5. How well does Pharmac understand child cancer and the system of care?

In the discussion document Pharmac asked four questions about the current system of care for children with cancer. Pharmac outlined a range of statistics and their understanding of the links between clinical trials and rule 8.1b. Submitters engaged closely with these questions and outlined how the rule is used in practice.

2.5.1 Many submitters said that rule 8.1b was an integral part of the child cancer system of care for all patients, not only those ones involved in clinical trials

Submitters were clear that the overall five-year survival rate for paediatric cancer in Aotearoa New Zealand was due to the overall system of child cancer care – of which rule 8.1b is an integral component. Submitters said that rule 8.1b was not only critical for children's access to clinical trials, but to the overall system of care. This links to the point explored in the overall themes in Part One, and in further detail below that rule 8.1b is not being used to fund the experimental drug that is part of clinical trials and is instead used to fund standard of care medicines also needed or potentially needed by the child enrolled in the trial.

The Child Cancer Foundation said that rule 8.1b was an integral part of a system that has achieved equity of access for children diagnosed with cancer and that the component parts of the system *"work in harmony to enable world-class outcomes to be achieved"*. They went on to say that the component parts of the system were rule 8.1b, shared care, national protocols, specialist treatment hubs, and no private provision of paediatric cancer treatment in Aotearoa New Zealand.

The National Child Cancer Network made similar points, stating that *"The whole children's cancer treatment system of care relies entirely on the provisions contained within rule 8.1b. Not simply for those enrolled on clinical trials"*. As well as many submitters stating rule 8.1b was funding medicines found on comparable countries' lists of funded medicines, the National Child Cancer Network said that the rule funds medicines that have recently been the subject of a (successful) clinical trial and are subsequently used to treat patients (including as part of subsequent trials for other medicines as the "standard of care" drug).

2.5.2 Many submitters said that rule 8.1b was required for those diagnosed with a paediatric cancer to access clinical trials and provided information about the operation of clinical trials for paediatric cancer

In response to the questions about the links between clinical trials and rule 8.1b, and how sensitive the clinical trials system was to any changes to rule 8.1b, submitters said that the continued ability for those diagnosed with a paediatric cancer in Aotearoa New Zealand to access clinical trials hinged on rule 8.1b.

As the consensus submission from paediatric oncologists and paediatric haematologists said: *"Our ability to continue offering paediatric clinical trials is dependent on rule 8.1b"*. The same language was used by Leukaemia and Blood Cancer New Zealand who said: *"Access to clinical trials for those with paediatric cancers is…highly dependent on patients being able to have timely access to treatments under rule 8.1b"*.

2.5.2.1 Many submitters said clinical trials were integral to continued good survival outcomes and equity in outcomes with other countries

Most submissions from clinicians and NGOs said that access to clinical trials facilitated by rule 8.1b was one of the reasons for comparable paediatric cancer survival rates with other countries. The National Child Cancer Network said that clinical trials were *"the vehicle through which survival rates have increased"* and said that in developed nations the option of clinical trials was considered *"standard of care"* for paediatric cancer. They submitted that given the reliance of clinical trials on rule 8.1b, if the rule were removed, children would no longer receive the most contemporary treatments and *"world class outcomes would be compromised…as the rest of the world continues to refine therapies and explore new agents"*.

The consensus submission from paediatric oncologists and paediatric haematologists said that *"we enrol 20-40% of paediatric oncology patients on clinical trials across disease groups including Māori, Pacific and patients living in rural areas"* and that disestablishment of rule 8.1b *"will decrease clinical trial activity"*. They also submitted that clinical trials do not only test the effectiveness of new treatments, but they have also *"driven efficiency of care"*. To illustrate this point, they referred to the example of a clinical trial which led to the reduction in the duration of chemotherapy for acute lymphoblastic leukaemias and lymphomas from 3.5 years to 2.5 years, which has significant benefits for patients, whānau and resources.

Some submissions from family and whānau echoed the explanations of clinicians and NGOs, saying that clinical trials were *"essential"* for the treatment of paediatric cancer and that children would not have access to those trials *"if they cannot get access to medicines through rule 8.1b"*.

Two submissions (from parents of a child who had been treated for cancer) explained that the clinical trial their child was enrolled in meant the treatment plan changed, with a reduced number of rounds of chemotherapy and reduced dose of radiation. They explained the substantial impact this change had on their child and said their hope for the future of cancer treatment, through clinical trials, was for advances *"not just in survival, but in survivorship"* because current treatments may create long–term side effects for children. This experience of long-term side effects, including the treatment itself leading to the death of a child, was spoken about in other family and whānau submissions.

The same submission said that given the reliance of clinical trials on rule 8.1b, any changes would *"take our paediatric oncology service out of the game – that our tamariki, even those who don't utilise the 8.1b provision, will not have access to the international trials that make a real difference to our children and their families"*.

Three of the four pharmaceutical supply companies that submitted (AbbVie, Johnson & Johnson, and Biogen) said that clinical trial participation in paediatric cancer in Aotearoa New Zealand was dependent on access to medicines through rule 8.1b, and that clinical trials had played a significant role in the progress of prognosis and survival rates for paediatric cancer.

2.5.2.2 Submitters said that there were several ways that rule 8.1b supported clinical trials in practice

Clinicians and NGOs explained that most clinical trials provided the novel drug, subject to the trial, for free and relied on rule 8.1b for the child to have access to those and other medicines required for participation in the clinical trial. Many clinicians and NGOs said that without ready access to the *'standard of care'* medicines through rule 8.1b children would not be eligible for clinical trial enrolment.

The submissions from the Children's Oncology Group, the New Zealand Association of Clinical Research (NZACRes), and several other clinicians and NGOs said that rule 8.1b supports international clinical trials in two ways. First, researchers need to ensure post-trial access to an equivalent treatment and second, clinical trials typically require the standard of care to be similar across the different countries where the trial is being conducted. They submitted that removal of rule 8.1b may mean less clinical trials because of the absence of the described guarantees.

This point was reinforced by Medicines New Zealand who said that the clinical trials dataset for 2013-2018 they had previously obtained from Medsafe indicated the majority of treatment focused paediatric cancer clinical trials in Aotearoa New Zealand were led and sponsored by not-for-profit/academic collaborative clinical trials. They understood that this has not changed since 2018 and the availability of funding through rule 8.1b was essential for participation. They submitted that it was *"important to note that in many instances the medicines funded for the trials through rule 8.1b are standard of care treatments elsewhere in the world"*.

Leukaemia and Blood Cancer New Zealand added that Pharmac's regular approval processes were *"incompatible with the clinical trial environment which is known for a fast turnaround…to continually build on new learnings"*.

The Cancer Society of New Zealand submitted that the 'hub and spoke' approach of the paediatric oncology national service (Starship and Christchurch hospital) can be viewed as a model for other parts of the health system. They said that, unlike in paediatric cancer care, access to clinical trials is not fairly distributed in other areas of the health system with most located in major centres and *"largely unattainable"* for people living rurally or in areas of high deprivation, as well as for Māori.

2.5.2.3 Submitters said new drug development for paediatric cancer was difficult and expensive, meaning there was low financial support from pharmaceutical companies and therefore a reliance on rule 8.1b to access clinical trial medicines

A few submitters said that there are limited incentives for pharmaceutical companies to invest in drug development for paediatric cancers. This is because paediatric cancers are rare, meaning there is a small population of people who will access any new drugs, which in turn means there are high costs to developing new drugs.

Brain Tumour Support New Zealand submitted that this high cost meant that there were lower profit margins for pharmaceutical companies for paediatric cancer drug development, meaning (as has been mentioned above) that many paediatric cancer trials do not have the financial support of pharmaceutical companies, instead being cooperative trial networks. They submitted that this lack of financial support from pharmaceutical companies meant trial investigators in Aotearoa New Zealand required rule 8.1b to access trial medicines. The same point was submitted by Rare Disorders New Zealand.

The Australasian Leukaemia and Lymphoma group submitted that another consequence of the high costs to developing new drugs for paediatric cancer was that *"very few medications used in paediatric oncology have a rigorous Phase 3 clinical trial or robust evidence"*. This means that promising treatments would be disadvantaged if they were subject to the same evidence requirements as adult medicines, when considering what to fund. Rule 8.1b means that this is not a concern and promising treatments can be used when beneficial, including as part of clinical trials.

These points were supported by one of the submissions from a pharmaceutical company. Johnson & Johnson said, *"pharmaceutical companies have a relatively small presence in New Zealand due to the relatively low investment in pharmaceuticals… thus Global pharmaceutical clinical trial investment in New Zealand is relatively low"*. This submission about low investment was also made by the pharmaceutical research and development company AbbVie.

2.5.2.4 Some submitters said there were benefits other than access to new therapeutics to clinical trial involvement (which is dependent on rule 8.1b)

One family and whānau submission said that if the ability to participate in clinical trials was lost, they expected that Aotearoa New Zealand would *"lose specialist doctors and nurses"*. A submission from a concerned individual stated that participation in clinical trials was *"vital"* for clinicians to access specialist training and maintain currency and clinical excellence.

The fear of losing expertise from Aotearoa New Zealand was also submitted by the Child Cancer Foundation who said that clinician fulfilment and satisfaction was important for retention and recruitment of clinicians. They said that if access to medicines through rule 8.1b was not available it would make clinical trials more challenging and could lead to *"clinician compassion fatigue"*.

2.5.2.5 Some submitters said clinical trials were also important for the treatment of other rare diseases

Rare Disorders New Zealand submitted that participation in clinical trials was especially important for those with rare disorders as it was often the only way to receive standard of care treatment. They submitted that they believe *"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"*.

2.5.3 Submitters generally agreed with the data used in the discussion document, but raised some questions

Some submitters said that Aotearoa New Zealand should be proud of the world-class care provided to those diagnosed with a paediatric cancer, which had the corresponding effect of comparable survival rates. A few family and whānau of those diagnosed with a paediatric cancer who submitted said that although the survival rates were comparable to other countries, Aotearoa New Zealand should be working to increase them further and keeping all avenues (such as rule 8.1b) open to enable that work.

A submission from a parent whose child had been treated for cancer said *"I think all cancer mums and dads would say – 80% isn't high enough. That 20% are our friends…the 3-year-old…who just missed out on turning 4…"*.

Outside of this general agreement that the discussion document had accurately recorded the general statistics of paediatric cancer, the following concerns were raised about the data.

a The AYA Cancer Network Aotearoa submitted that while the discussion paper was correct for the paediatric population (aged 0-14 years), it was not accurate for people with paediatric cancers. 'Paediatric cancers' are not defined by the age of the patient, but by the type of cancer. The AYA Cancer Network Aotearoa submitted that AYA survival rates for paediatric cancers were lower than for children with paediatric cancers. The example they gave was a recent assessment of five-year survival rates for osteosarcoma which was *"just 51% for adolescents compared to 80% for children"*.

b The Child Cancer Foundation submitted that it was difficult to assess Pharmac's understanding of the use of rule 8.1b because of the way the data was presented. For example, the cost figures included 18- to 25-year-olds who they submitted would be seen as adults and not eligible for access to medicines through rule 8.1b. They further submitted that the figure of 7% presented as the proportion of paediatric cancer patients that access medicines using rule 8.1b was *"actually much higher"* and therefore removal of the rule would have a greater cost than envisaged.

c The Brain Tumour Support Trust New Zealand and Rare Disorders New Zealand said that the overall survival rate statistics *"mask the relatively dire survival rate for CNS (central nervous system) 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"*[6] and that the 73.5% survival rate also misrepresents the situation for aggressive sub-types of paediatric brain tumours. This point was reiterated by a submission from a family who said the cancer their child died from had a 30% survival rate.

d Several NGOs noted that there was not any statement about adult cancer survival rates, which unlike for paediatric cancer are not comparable to other countries (several submissions said that a large part of this difference was better access to medicines for children).

e Leukaemia and Blood Cancer New Zealand said that they had some issues with data used in the discussion document, including data from 15- to 24-year-olds being used to estimate the costs of paediatric cancer medicines as many 15- to 24-year-olds do not have a paediatric type of cancer. They submitted that therefore they were not being treated by child cancer services and were not accessing medicines under rule 8.1b.

f Patient Voice Aotearoa said that there was no inclusion of five-year survival rates for children with rare disorders, and they would like to know how Aotearoa New Zealand compared to other countries in those statistics.

6 The submission referenced "NCCN paper 2015-2019".

2.6. How effective is rule 8.1b in terms of achieving the best health outcomes?

In the discussion document Pharmac asked two questions about the effectiveness of rule 8.1b in achieving best health outcomes. Pharmac stated that although Aotearoa New Zealand has good outcomes for those diagnosed with a paediatric cancer, it was not clear whether these good outcomes were dependent on making paediatric cancer treatments available through 8.1b.

2.6.1 Most submitters said that access to medicines is needed for good outcomes, and rule 8.1b provides that access

Most submitters who specifically responded to question five (To what extent are good health outcomes for those diagnosed with a paediatric cancer in New Zealand dependent on making paediatric cancer treatments available through rule 8.1b?) agreed that the currently experienced good survival outcomes were dependent on access to medicines through rule 8.1b. One parent of a child with cancer said, *"this is what the provision of world-class health care looks like"* while another said *"The evidence – benchmarked internationally – speaks for itself"*.

The consensus submission from the paediatric oncologists and Paediatric haematologists said, *"Change to 8.1b is a fundamental threat to the high performance of child cancer"*. Leukaemia and Blood Cancer New Zealand expanded this point by saying that *"Paediatric cancer survival success can be attributed to a few simple, but critical, factors: access to internationally recognised medicines/ treatments (without the restrictive financial boundaries applied to adult population treatment); participation in clinical trials; national consistency in service provision; wrap-around supportive care for children and their whānau"*.

The National Child Cancer Network submitted that while many of those diagnosed with a paediatric cancer have excellent health outcomes and do not require access to medicines via rule 8.1b, *"this is because earlier cohorts of children did access medication through 8.1b, and over time these medications have become incorporated into those that are publicly funded"*.

This submission about rule 8.1b being an integral part of good outcomes as a component part of a system of care was also made by some family and whānau submissions. One parent of a child who had been treated for cancer said, *"'Poorer outcomes' doesn't really convey the gravity of what losing the care environment the rule provides would actually mean…while many…patients like my [child] may not appear to benefit from the rule directly, the system of care it underpins provides better and equitable outcomes for all of them"*.

Medicines New Zealand submitted that rule 8.1b *"is likely to be the determinative factor in the comparability of outcomes with our peer nations"*. The Child Cancer Foundation took a slightly different approach, submitting that rule 8.1b was a *"component part"* of the system of care which has meant there are comparable outcomes with other countries. They submitted that this was a case of *"the whole being greater than the sum of its parts"*, and access to medicines through rule 8.1b was a *"key component"* of the whole.

A few submitters made the inverse point: that without rule 8.1b survival outcomes would get worse. The Head and Neck Cancer Support Network, Tuberous Sclerosis Complex New Zealand, Cheekee Hero Charitable Trust, Friedreich Ataxia Research Association New Zealand (FARA NZ), atypical Haemolytic Uremic Syndrome (aHUS) and the New Zealand Pompe Network said *"without [rule 8.1b] outcomes would get worse"*, and a young person who lost their sibling to cancer said in relation to this *"my question would be why take that [good/equitable outcomes] away?"*.

A few submitters compared outcomes for paediatric cancers with outcomes for adult cancers in Aotearoa New Zealand. Leukaemia and Blood Cancer New Zealand said, *"Ending 8.1b would significantly reduce the number of available paediatric clinical trials, which would worsen outcomes (as demonstrated in the adult population)"*. Cure Our Ovarian Cancer also made this submission, saying that the difference in survival rates between some paediatric cancers and adult cancers was *"mostly due to treatment access and clinical trials for tamariki that are facilitated by rule 8.1b"*.

Families and whānau said that rule 8.1b was crucial to maintaining the current survival rates, and that access to medicines through rule 8.1b had saved their child's or family member's life:

> ❝ Starship Auckland having the discretion to access 8.1b saved our [child's] life ❞
>
> ❝ My [child] would be dead. That is enough for me ❞
>
> ❝ What contributed to her being disease free? Living in a country that has world leading paediatric oncology services, having access to an international study and the absence of financial or other barriers preventing her from getting the care she needed ❞
>
> ❝ [Removal of rule 8.1b] almost certainty means more children will die, that we will get to spend less time with the children in our lives ❞
>
> *Submissions from whānau Māori*

A final point made in both the consensus submission from paediatric oncologists and paediatric haematologists and the submission from the National Child Cancer Network relevant here is that currently some paediatric cancer medicines not on the Pharmaceutical Schedule are accessed through pharmaceutical company compassionate access programmes. They submit that these may not be visible to Pharmac or appear on any list of *"all publicly funded paediatric cancer treatments"* but are considered 'standard of care'. They submitted that if compassionate access is lost, for whatever reason, a funding avenue would be required.

2.6.2 Many submitters said timely or immediate access to medicines was imperative for the treatment of paediatric cancer

All submitters who responded directly to question six (Is timely access to paediatric cancer treatments more important than timely access to other medicines or for other populations? If so, why?) said that timely access to paediatric cancer treatments was imperative, and some made submissions that timeliness may be more important for paediatric cancer than other diseases.

The Child Cancer Foundation, along with other NGO submitters, said that paediatric cancer frequently presents acutely and *"there is little time for extensive consultation on access to medicines"* which is unlike other childhood conditions. They submitted that in general the longer a child waited for medicines the sicker the child became, and therefore there was a higher likelihood of complications occurring. They said that they do *"not dispute the fact that Rule 8.1b creates inequality of access to certain therapeutics between some children with a cancer diagnosis and those with other rare disorders"*. However, they submitted that other disease presentations *"allow more time to thoroughly evaluate the risks/benefits, financial cost and likely outcomes of various treatment options"*.

The AYA Cancer Network also said that timely access was important because childhood cancers tend to grow and progress more rapidly than adult cancers. They said that while the diagnosis timeline for AYA was longer than for children, it was also true of cancers for AYA.

The National Child Cancer Network made similar points, saying that cancer in children was *"almost always aggressive and rapidly growing"* requiring treatment to start within a short timeframe. The consensus submission from paediatric oncologists and paediatric haematologists agreed with this point saying that it was *"pertinent to note"* that the formation of adult cancer was usually a *"step wide process"* in contrast to the shorter time period of childhood and adolescence cancer growth which was rapid, with high-risk presentations from initial diagnosis and relapse.

Submissions from family and whānau shared their experiences of what 'a short timeframe' meant for their children:

> ❝ [My child's treatment changed] an hour after we got the MRI…it needed to be changed that very day ❞
>
> ❝ Within the space of a week we went from thinking she might need glasses to being at the hospital…hearing the news that she has cancer, and hearing the news that it was incurable…surprised and pleased at how quick treatment was there and available ❞
>
> ❝ [Our child's] condition rapidly deteriorated… we needed to act fast as the tumour continued to grow and was pushing further on [their] brainstem… causing [them] to become weaker and weaker every day ❞
>
> *Submissions from whānau Māori*

Submissions from family and whānau also pointed out the differences between paediatric cancer and adult cancer necessitating a different approach to timely access. One such submission referred to statements made by Cancer Research UK and journal articles which said that childhood cancers were *"not small adult tumours, but instead show unique genetic changes and thus diagnoses and therapies should be treated differently to adult cancers"*.

The consensus submission from paediatric oncologists and paediatric haematologists expressed their *"grave concern"* about any changes to rule 8.1b (including devolving to NPPA instead of rule 8.1b) leading to longer lead-times for ordering and supply of medicines meaning that they would not be able to be given to children in a reasonable timeframe. They gave the specific example of nelarabine for T-ALL which was used infrequently and had a shelf expiry that meant it was not usually kept in stock. They submitted that the turnaround using rule 8.1b was *"already tight"* and if there was any administrative burden between the return of results indicating the need for the medicine and ordering it due to a new application process there would be *"unacceptable delays in administering medicines"*. The Cancer Society of New Zealand also said they had *"substantial concerns"* if paediatric cancer medicine approval had to go through a similar pathway to adult cancer treatments.

The National Child Cancer Network submitted that there are current issues with accessing medicines not directed at the cancer but used to treat those diagnosed with a paediatric cancer in a supportive care capacity. They submitted that when unfunded they are accessed via the NPPA mechanism and there are *"frustrations and inconsistencies reported by paediatric oncologists and paediatric haematologists"*. They said that while not optimal for non-cancer related medicine access "*[it] would be viewed as untenable for accessing cancer-related medicines"*.

A submission from a parent provided their experience with this happening. Their child requires a medicine not directed at the cancer and each course needs to be applied for. For each course *"there is always a delay in approval and always a gap between courses"*.

Submissions from other families and whānau supported this point, saying that the system needs *"access without delays"* and any changes to the current system that required clinicians to make applications or seek approval would see them *"navigating red tape, paperwork and failing applications"* which would increase pressure, clinical demands, compassion fatigue and impact on the time clinicians could spend with patients and families and whānau. The same submission from a family said that if applications needed to be made to access medicines, delays would be *"inevitable."*

Another family submission asked, *"will children pass away while waiting for decisions to be made?"*. There was one pharmaceutical company that made a submission about timeliness. Johnson & Johnson said that any changes to rule 8.1b would compromise speed of access, noting that the current average time from a recommendation from the Pharmacology and Therapeutics Advisory Committee (PTAC) to funding a medicine is 40 months.

2.6.3 Some families and whānau submitted that the existence of rule 8.1b meant, on the whole, they could focus on their child, not have to advocate or fundraise for appropriate treatment, and feel comforted that they did everything they could for their child

The section below describes what submissions from families and whānau said about how the availability of medicines through rule 8.1b impacted them.[7]

Families and whānau of those diagnosed with a paediatric cancer, including parents, siblings, aunts, uncles, grandparents, and godparents, spoke about the impact of their child's cancer diagnosis on the whole family and the *"relief"* felt when they discovered the best possible treatments were available in Aotearoa New Zealand. Many of the family and whānau stories included detail about what day-to-day life was like during the time of their child's treatment, and how it was a diagnosis *"for the whole family"*.

Families and whānau submissions talked about one parent giving up work to care for their child full-time. Others had family and whānau members give up work, move houses or areas to support them, and provide emotional and financial support to the parents, and siblings of the child with cancer. Some of them spoke about the financial strain put on their family due to the loss of income and the costs associated with their child's treatment, outside of direct treatment costs (including travel and accommodation).

Many families and whānau said that rule 8.1b was one less thing to worry about. The parent of one child who had been treated for cancer said that it was *"a blessing we don't have to worry about how to access treatment on top of [everything else] … when we are already running on empty"* after saying the following about daily life with a child being treated for cancer:

> ❝ …[I] don't want to see whānau having to leave their children's bedside to make their child's voice count or to run fundraising campaigns to get the medicine their child needs…all you can do is survive. Your own health and wellbeing, the wellbeing of the other tamariki in your family suffers, it is 24/7. Your life becomes consumed by catching vomit and the inevitable laundry that goes with it, it is rushing your child to the toilet while hooked up to multiple IV infusions, it is driving to ED in the night, it is monitoring temps, giving meds, it is cajoling them to eat and when it fails managing nasal gastric feeds. You spend your time trying your best to minimise the impact…on your child. It is cuddles, it is playing snap over and over again, it is trying to keep them calm for dressing changes and needles, it is keeping on top of appointments, it is seeing other members of your family by video call because you have to be isolated, it is battling traffic to get to treatment by 7am, it is handing your child over for their 50th general anaesthetic, it is practicing breathing exercises for a CT scan, it is sleeping on a murphy bed away from home, it is all consuming and relentless ❞
>
> *Family submission*

Other submissions from families and whānau on this point included:

> ❝ You're already in the worst situation you can be in – what you need is support from everyone, a good health system, and that means there is treatment options and something you can do. And that is rule 8.1b ❞
>
> ❝ [If rule 8.1b is removed] Will you now be placing more stress on both families and clinical staff? Yes ❞
>
> ❝ A child being diagnosed with cancer is a stressful and turbulent time for families… child cancer is a 'diagnosis for life' and sucks everyone in the family in ❞
>
> ❝ I can't tell you the relief I felt when [the oncologist] said that for childhood cancer we would get world-class care and access to the best cancer [medicines] without having to apply for additional funds…the relief of that was so immense… ❞
>
> *Family submission*

Families and whānau, especially those whose children had died from cancer, also submitted that having rule 8.1b meant that they had a *"sense of peace"* knowing they had done everything possible for their child, and that they would not have had different and better opportunities for treatment elsewhere.

> ❝ There is solace in knowing that your child was given a fighting chance…there is comfort in hope and by providing access to treatment, rule 8.1b is a gateway to hope ❞
>
> ❝ …if there was a treatment option that was not available in NZ but had shown promise…overseas, I would have carried this grief with me for the rest of my life ❞
>
> ❝ We have few regrets, because we have done everything we can ❞
>
> *Family submission*

Families and whānau also submitted that rule 8.1b enabled paediatric oncologists to spend time with their patients and not lobbying for medicines to make it onto the Schedule *"…we want our oncologists to be out there doing the mahi of saving lives"*.

2.6.4 A few submitters said timely access to medicines was also important for rare diseases and AYA with cancer

A few submitters said that timely access to medicines was important for other diseases. The AYA Cancer Network said rapid access to medicine was also important for AYA with cancer because their cancers tend to grow and progress more rapidly than adult cancers. Some other NGOs, particularly those representing other rare diseases, said that rapid access to medicines was also important for other diseases. It said:

> ❝ 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. ❞

7 Part 2.8.7 below will outline the related point about what submitters said about the possibility of rule 8.1b being replaced with a system that required families and whānau to self-fund paediatric cancer medicines.

2.7. Does the current policy support efficient and sustainable use of available resources?

In the discussion document Pharmac asked four questions about whether the current policy supported efficient and sustainable use of available resources. Pharmac provided some estimates of the total cost of paediatric cancer treatments and outlined some of their concerns about the costs of future paediatric cancer treatments.

2.7.1 Many submitters said that the financial investment in rule 8.1b medicines was small, not expected to grow significantly, and resulted in world-class outcomes

Many submitters said that the financial spend on medicines accessed through rule 8.1b was very small, particularly given that treatment usually leads to the child being cured (this point is explored more below). These submitters frequently referred to the statement in the discussion document that the total cost of paediatric cancer treatment for people aged under 25 was approximately $5.5 million and less than 1% of the CPB.

In the submission from the Child Cancer Foundation, their CEO, Monica Briggs, was quoted, *"We currently have a system that achieves world-class outcomes for very little resource; why would we want to change this?"* This was echoed by the consensus submission from paediatric oncologists and paediatric haematologists who said that the *"modest financial impact needs to be balanced against the risk to world class paediatric cancer survival, the shifting of financial burden to fund medicines to whānau, the creation and exacerbation of inequalities…and avoiding the aftermath for families tormented by the death of children where a standard of care medicine was unavailable"*.
The submission from the Child Cancer Foundation went on to say that the average cost of medication for a child with cancer was around $8,000 per patient, under the age of 25. They submitted that given most *"go on to live healthy and productive lives"*, it *"would appear to be a 'value for money' investment"*.[8]

The National Child Cancer Network submission also stated that the *"financial burden of rule 8.1b was very small given that it is "worth remembering… numbers"*. They submitted that there are 25,000 adults diagnosed with cancer each year, and 153 newly diagnosed children, *"only 30% of whom will require medication through rule 8.1b, and of that only 30% for a more 'bespoke' reason and not standard of care"*.

The submission from Patient Voice Aotearoa in response to questions about efficient and sustainable use of resources said that they *"view the growth of modern medicine to treat children's cancer as a welcome development, rather than a concerning move forward… Each modern medicine is a lifeline to children… [and] should be celebrated, rather than viewed as a cost and a burden on the taxpayer"*.

The Brain Tumour Support Trust New Zealand submitted similarly, saying that *"Rather than fear the costs of new treatments…Pharmac should consider the revolutionary health benefits that many of these treatments can provide"*.

Submissions from family and whānau also talked about the low financial spend on paediatric cancer medicines, and those accessed through rule 8.1b. One submission said that *"arguments of cost are weak"* given the less than 1% spend from the CPB. A family member of a child who had died from cancer said *"it's purely a budget measure, which makes me feel a bit ill to be honest"* about the fact rule 8.1b was being reviewed. Another parent of a child treated for cancer said they recognise that resources are finite and that there had been recent increases in Pharmac's budget, however *"… the amount of money spent on paediatric cancer is a very small proportion and it produces fair outcomes, whereas removing rule 8.1b will produce a deeply unfair situation for children and families"*.

Some submitters talked about whether the cost of rule 8.1b would increase. A few thought that it would, while a few others said that while they thought that was possible it was not likely to be significant.

The National Child Cancer Network submitted that it was likely that longer term costs were going to rise, especially given precision medicine technology which identified druggable targets. They submitted, however, that the population requiring those is very small. This view was supported by a pharmaceutical company, Johnson & Johnson, who said that it was *"important to note that new innovative precision, cell and gene therapies which may be considered 'high initial cost' will be:*

(i) targeted or precision therapies – eliminating futile treatment
(ii) one-off treatments, rather than, treat to progression
(iii) potentially curative therapies which improve their overall clinical effectiveness
(iv) improved cost effectiveness over a lifetime."

Medicines New Zealand submitted that *"claims regarding costs of future medicines would appear not to be robustly grounded in comprehensive research and analysis"* as a result of limited consultation with the pharmaceutical sector.

They suggested the *"adoption of an approach of strategic relationship setting and enhanced stakeholder engagement with the pharmaceutical sector…ensuring that New Zealand is able to maintain paediatric oncology outcomes on par with relevant OECD comparator nations"*.

The consensus submission from paediatric oncologists and paediatric haematologists said that it was *"difficult to predict"* whether there would be an increase in costs of medicines accessed through rule 8.1b. They said they predicted a *"modest"* increase and that a bigger factor for consideration than increased cost of individual medicines was the potential for pharmaceutical companies to end the compassionate supply of some standard of care paediatric cancer medicines (which was outlined above).

2.7.2 A few submitters said that the use of rule 8.1b was carefully considered

A few submissions said that the current use of rule 8.1b was carefully considered by clinicians and that it was not treated as an *"open chequebook"* (Child Cancer Foundation submission).

The paediatric oncologists and paediatric haematologists' submission said that they felt it was *"important to note…that prescribing of medications accessed under rule 8.1b historically has been careful and judicious"*. They reviewed the past five years of prescribing under rule 8.1b (which was 242 instances) and concluded that they *"would feel comfortable justifying the use of every medication as international best practice"*.

This submission was also made by the National Child Cancer Network who said that *"specialists are making decisions on available evidence…often despite significant pressure from desperate families and whānau, who may have read about or even been advised by overseas clinicians to follow such a pathway"*.

The Brain Tumour Support Trust New Zealand submitted that rule 8.1b was used too cautiously by clinicians.

There was one submission from a parent of a child treated for cancer that said their experience was that *"paediatric oncologists are far more protective of parts of the health budget that they control than someone on the outside might reasonably expect, and this protectiveness extends to many different spend areas… not just pharmaceuticals"*.

8 The submission did query the data presented in the discussion document about cost because it included 18- to 25-year-olds who they submitted would not be eligible to access medicines through rule 8.1b.

2.7.3 Some submitters said that paediatric oncologists are best placed to make decisions about what medicines to administer

Some submitters (including the Child Cancer Foundation, the AYA Cancer Network, the submission from the AYA Cancer Consumer Advisory Group and the National Child Cancer Network) said that paediatric oncologists were best placed, due to their expertise, to make decisions about the treatment of paediatric cancer. They also noted that these clinicians were already making decisions under rule 8.1b, and it was unlikely that sufficient expertise could be found elsewhere to make different or better decisions about what should be funded.

> “ We believe that the best treatments for paediatric cancers should be available to be prescribed by doctors without the rigmarole of the standard approval process. ”
>
> *AYA Cancer Consumer Advisory Group*

> “ ...we believe the motivations of clinicians who, in our view, are the best decision makers and perhaps Pharmac's best gatekeepers ”
>
> *Child Cancer Foundation*

2.7.4 A few submitters said Pharmac should have more oversight of expenditure on medicines accessed through rule 8.1b

A few submissions said that it would be appropriate for Pharmac to have more oversight of the operation of rule 8.1b.

The consensus submission from paediatric oncologists and paediatric haematologists said that the track record for the use of rule 8.1b *“reflects good management of financial risk”* but acknowledged that there were future risks to mitigate. They submitted that the current process *“does not provide the visibility expected by the public and Pharmac...[and] although we feel strongly that access to medication...has been judicious and appropriate... we appreciate the need to make our robust process more visible”.*

The National Child Cancer Network suggested that for clarity and visibility of the process for using rule 8.1b, Pharmac could put a mechanism in place to see the rationale for clinical decisions to use the rule. This was supported by the AYA Cancer Network Aotearoa and one submission from a family.

2.7.5 Many submitters said that cancer treatment for children is usually curative and therefore an efficient use of resources

Many submissions across all types of submitters talked about the curative nature of paediatric cancer treatment, stating that investment in curative treatment for children was a good investment given it was short term and likely to result in many years of life following treatment.

A few submitters said Pharmac should be taking Quality-Adjusted Life-Years (QALYs) into consideration. However, one submission from a pharmaceutical company, Johnson & Johnson, said that using QALYs as an assessment can *“drive inequity as it poorly represents paediatric patients and differences in ethnicity”.*

It is worth noting that some submitters expressed discomfort at trading different groups off against one another when discussing these issues, for example considering whether those diagnosed with a paediatric cancer were more *“deserving”* of funded medicines than other groups. As a submission from a parent said that, for other conditions lifelong treatment is needed, but they are only making that point *“if we must place an economic value going forward”.* This discussion is picked up below where submissions about equity issues are summarised.

Many NGOs submitted that the *"return on investment"* for childhood cancer treatment was high. This included the Child Cancer Foundation, Leukaemia and Blood Cancer New Zealand, Brain Tumour Support Trust New Zealand and Rare Disorders New Zealand. They said that most children who are treated for cancer are treated for *“around two years”* and therefore costs were finite in contrast to many other chronic diseases where treatment can be lifelong. The consensus submission from paediatric oncologists and paediatric haematologists also said that there was a *“greater return on investment”* in investing in child health (compared to adult health).

The Child Cancer Foundation submitted that they would like to see QALY data from Pharmac, otherwise there was little evidence, other than speculative, to be able to discuss whether investment in paediatric cancer medicines should be prioritised over investment elsewhere.

There were two submissions from family and whānau that said resources spent on treating childhood cancer should be prioritised over diseases they submitted were preventable.

2.7.6 Many submitters said Pharmac should take a broad approach to considering the benefits of funding paediatric cancer medicines

In response to our set of questions in the discussion document about whether rule 8.1b was an efficient and sustainable use of resources, many submitters said that they thought a broader approach to considering the benefits of funding medicines should be taken.

Submissions from the Head and Neck Cancer Support Network, Tuberous Sclerosis Complex New Zealand, Cheekee Hero Charitable Trust, FARA NZ, aHUS and the New Zealand Pompe Network said that *“all social determinants of health”* should be taken into account, including assessment of medical, economic and social costs incurred if medicines were not funded.

The Child Cancer Foundation submitted that if Pharmac took a values and wellbeing-based approach to this issue it would consider the broad support provided to patients by the rule and the reduction in overall health system cost.

Medicines New Zealand echoed this point, saying that *"the central theme of the consultation seems to be costs of the rule rather than health outcomes, societal benefits and long-term benefits that come from innovations in treatment"*.

Some families and whānau and NGOs submitted that as well as the economic contribution those diagnosed with a paediatric cancer would go on to make, the impact on families and whānau of the loss of a child to cancer should be considered.

2.7.7 Some submitters spoke about the investment in medicines in Aotearoa New Zealand being very low compared to other countries and the impact this had on the use of rule 8.1b

Some submitters said that Aotearoa New Zealand's CPB was very low compared to other countries with the impact that a high number of cancer medicines that are approved and funded in those countries are not in Aotearoa New Zealand. Submitters said that this accounted for part of the reason why rule 8.1b is needed for paediatric cancer – and that if the CPB was higher many of the medicines accessed through the rule would be on the Schedule. This was referenced in submissions from all types of submitters.

The consensus submission from paediatric oncologists and paediatric haematologists referred to a 2022 report of Te Aho o Te Kahu, the Cancer Control Agency that identified 18 cancer medicines of "*substantial clinical benefit"* funded in Australia that were not funded in Aotearoa New Zealand. They further stated that to be included in the 18 the cut off was *"strident"*, suggesting there was an argument that the list could be longer.

One submission from the parent of a child treated for cancer explained that when her child was diagnosed with a rare form of cancer there were only ten publications reporting the efficacy of therapeutics against the type of cancer. In the subsequent eight years there has been a further 21 publications reporting potential therapeutics. Of those 21, only two (imantinib and dasatinib) have been put on the Pharmaceutical Schedule and a further three (bevacizumab, sorafenib and pembrolizumab) have been accessed through rule 8.1b in Aotearoa New Zealand.

Patient Voice Aotearoa said in disputing the framing of question 10 (How could we assess what value paediatric cancer treatments provide against other medicines that could be funded with the same money?) that an appropriate question would be *"What would the value be to New Zealand society if Pharmac was appropriately funded to cater for the medicinal needs of those in the population who require it?"* They submitted that they were not arguing for every medicine to be funded, however submitted that it was widely accepted that Pharmac needed substantially more funding.

One submission from a concerned individual stated that *"8.1b would not be needed if medicines were properly funded in New Zealand"*, while another said, *"In New Zealand we have the worst access to drugs in the developed world – with a correspondingly low drug budget"*.

2.7.8 A few submitters said concerns about significant expenditure increases as a result of CAR-T cell therapy were unfounded

A few submitters responded to the reference to CAR-T cell therapy in the discussion paper. They submitted that there should not be a high level of concern about increased costs because of this technology, with some explicitly making the point that it had never been accessed through rule 8.1b.

The National Child Cancer Network said that the therapy had been used three times for children in Aotearoa New Zealand in the past four years, and each time had been accessed through the High-Cost Treatment Pool (funded by Health New Zealand/Te Whatu Ora). They submitted that they were aware of the large number of early phase international trials with this therapy for solid tumour groups, however, did not believe there was a *"high likelihood of fiscal threat in the paediatric setting in the foreseeable future, if ever"*. They submitted that concern in this area would be *"highly speculative and assuming of clinical benefit or impact that is significantly ahead of where the field is at"*.

The consensus submission from paediatric oncologists and paediatric haematologists submitted similarly, saying that *"we feel strongly that [concern for rapidly increasing use of CAR-T cells] is unfounded"*. They submitted that there were *"few signals"* of meaningful clinical activity in the area of paediatric cancer let alone reaching the threshold as an accepted standard of care. They did submit that there was a clear exception to this, which was the use of CAR-T cells for relapsed/refractory B cell acute lymphoblastic leukaemia. They noted that the three children who had received this treatment (as referred to by the National Child Cancer Network in their submission) had been granted funding through the High-Cost Treatment Pathway Pool.

Two submitters, Leukaemia and Blood Cancer New Zealand and the AYA Cancer Network Aotearoa, said that potential concerns about the costs of CAR-T cell therapy were broader than only paediatric cancer and should be considered outside of the review of rule 8.1b.

2.8. Does the current policy support equity?

In the discussion document Pharmac asked five questions about the whether the current policy supported equity. While submitters provided significant and useful information across all of the questions, the topic of the highest volume of submissions was in response to this question.

2.8.1 Many submitters said that rule 8.1b was an example of a health service that has delivered equity

Many submitters said that while rule 8.1b may be inequitable in access between those diagnosed with a paediatric cancer and other groups needing medicines, it had created equitable outcomes within and between those diagnosed with a paediatric cancer in Aotearoa New Zealand and other comparable countries. The Child Cancer Foundation said, *"Paediatric oncology services in Aotearoa/New Zealand are a shining example of what the provision of health services that delivery equity of access look like".*

Some submitters said that paediatric cancer was one of the only areas of the health system in Aotearoa New Zealand that had achieved equity with comparable countries, between Māori and non-Māori, Pacific and non-Pacific, and across location and socio-economic background. They said that *"without significant budgetary impact"*[9] rule 8.1b *"is actually the very thing that has allowed us to produce equitable paediatric cancer outcomes".*[10]

One family submission said, *"the most critical aspect of the rule is that it allows removal of inequities [and] ensures [the] same standards by guaranteed access at no cost to families".* Another said *"the only way that we continue to achieve equity in childhood cancer is by fully funding necessary medicines, otherwise, as with adult cancers, Māori and minorities will have worse outcomes…"*

2.8.2 Some submitters said that children should be prioritised over adults in health spending

Some submitters said that in line with te ao Māori concept of He Taonga Te Tamariki and with Aotearoa New Zealand's domestic and international legal obligations, children should be prioritised over adults in health spending. Specifically referred to by submitters were obligations to Te Tiriti o Waitangi, the United Nations Convention on the Rights of the Child (which New Zealand has ratified), the United Nations Convention on the Rights of Persons with Disabilities (which New Zealand has ratified), the International Covenant on Economic, Social and Cultural Rights, the Pae Ora (Healthy Futures) Act 2022 and the New Zealand Bill of Rights Act 1990.

The consensus submission from paediatric oncologists and paediatric haematologists said that there were *"interdependent benefits and ethical obligations of investing in child health"*. They said that these included:

a. commitment to equity, because when priority is put on child health, it can contribute to evening out group-based disparities later in life
b. the State's duty of care to children because they cannot advocate for themselves or fully participate in democratic processes
c. the special status of children under the UN Convention of the Rights of the Child
d. that paediatric cancer *"sits at the confluence of many disadvantages"*. They submitted that these disadvantages included (but were not limited to):

    i. under-investment in children's health per head of the population
    ii. less research for rare diseases in children compared to other diseases
    iii. less drug development for those diagnosed with a paediatric cancer as the economic motivations were not there for pharmaceutical companies and cancer drug development was *"even more risky in paediatric age ranges"*.

A Māori parent whose tamariki had died from cancer said they *"totally get all parts of society and community…need support…but I think this is for the greater good of our most vulnerable part of the community, so it really doesn't make any sense [to change or restrict access to paediatric cancer medicines]"*. Another submission from a Māori parent said, "*As a society we can only be judged by how we look after our most vulnerable – those diagnosed with a paediatric cancer are some of our most vulnerable in society – not just them, but the whole whānau".*

Patient Voice Aotearoa registered their opposition to the question in the discussion document about how Pharmac should assess spending on childhood cancer medicines against other medicines (question 6). They submitted that the question should be *"How can we lift older people to the same level of medicine access as children who are accessing medicines under rule 8.1b?"*

2.8.3 Many submitters said that children's diseases should not be traded off against one another

Several submitters said that different children's diseases should not be traded off against one another when considering funding of medicines. Patient Voice Aotearoa communicated this submission with a quote from Judge Andrew Becroft from when he was the Children's Commissioner of Aotearoa New Zealand:

> ❝ …I don't want a situation to descend into playing one illness off against the other. And children, with their illnesses, being bargaining chips when they're facing life-threatening conditions. All children in New Zealand society, surely, can have access to lifesaving treatment. That must be the starting point ❞
>
> *Patient Voice Aotearoa referred to this quote from Judge Andrew Becroft (as Children's Commissioner)*

Some family and whānau submissions agreed with this general submission:

> ❝ I think Pharmac should look into better ways to fund children with rare disorders rather than changing an effective system within paediatric oncology ❞
>
> ❝ You should not be comparing cancer treatments to other medications – they need their own rule ❞
>
> *Family submissions*

9 Submission from Breast Cancer Aotearoa Coalition
10 Consensus submission from paediatric oncologists and paediatric haematologists.

2.8.4 Many submitters said that rule 8.1b had achieved equity in outcomes between tamariki Māori and non-Māori children, and Pacific children and non-Pacific children

All types of submitters (including families, whānau Māori and the submission from a Pacific family) spoke about the widely known inequity for tamariki and whānau Māori across health outcomes in Aotearoa New Zealand and expressed that paediatric cancer treatment should be looked to as an example of how equity for Māori in health outcomes can be achieved. The same point was made about inequity between Pacific children and non-Pacific children.

Clinicians and NGOs said that changes to or removal of rule 8.1b would have a negative impact on equity between non-Māori children and tamariki Māori and Pacific children. One clinician said that *"building upon vital work to continue to remove inequities for Māori and Pacific tamariki with cancer will only be possible with the continuation of 8.1b"*.

2.8.5 A few submitters said that for Pharmac to meet its obligations under Te Tiriti o Waitangi and the Te Pae Ora (Healthy Futures) Act 2022 it should retain rule 8.1b

In addition to the submissions from whānau Māori outlined in the section above about Pharmac's obligations under Te Tiriti o Waitangi and the Pae Ora (Healthy Futures) Act 2022, a few other submissions said that removal or changing rule 8.1b would contradict Pharmac's obligations in that regard.

Patient Voice Aotearoa said that *"Pharmac cannot claim to be committed to Ōritetanga (equity) under Article Three or be Te Tiriti-led"* if change or removal of rule 8.1b went ahead, because it would *"see the lives of Māori children with cancer being cut short"*. The Child Cancer Foundation submitted that they had *"...already been asked if removal of rule 8.1b could be the subject of a Waitangi Tribunal determination should a family be motivated to make a claim"*.

In reference to Pharmac's obligations under the Pae Ora (Healthy Futures) Act 2022, Medicines New Zealand said that because achieving *"best health outcomes"* was a *"central driver of its statutory objective"* rule 8.1b should be retained as that is what it achieved. They submitted that as Pharmac is currently delivering its statutory goal for paediatric oncology, *"why risk [falling behind] now?"*

2.8.6 Some submitters said that rule 8.1b had achieved equity in outcomes between children from different socio-economic backgrounds and geographical locations across Aotearoa New Zealand

Some submissions said that rule 8.1b had also achieved equity between children of different socio-economic backgrounds and geographical locations.

The submission from one family of a child treated for cancer said that the children, who have been treated for cancer, and their families, are incredibly unique, and have *"lived experiences only those who have been through child cancer can know about"*. They said that rule 8.1b *"allows us all to have similar lived experiences because access to medication is the same for everyone"*.

However, the consensus submission from paediatric oncologists and paediatric haematologists talked to the financial burdens under the current system (indicating some inequities based on socio-economic backgrounds do exist). They referenced research that was currently underway about the experiences of family and whānau in Aotearoa New Zealand whose child has received treatment for cancer. They submitted that the interim analysis has revealed that *"the cohort suffered a median $18,000 reduction in household income, with 13% requiring a loan to cover costs, and 70% describing additional financial costs of care that were not met by Governments or NGOs over the first year of diagnosis"*.

2.8.7 Some submitters were concerned that if rule 8.1b was removed or changed a system at least partially reliant on self-funding would be inevitable

Some submitters, across all submitter types, were concerned that without rule 8.1b, or if changes were made to rule 8.1b, a *"two-tiered"* system would be created for paediatric cancer treatment in Aotearoa New Zealand.

The Cancer Society of New Zealand submitted that *"It would be of great concern if a change to the funding regime created an incentive for private paediatric cancer treatment providers to be established – this would create a two-tiered system; drive inequities; and also have a considerable adverse flow on consequences to cancer support providers"*.

Submitters said that this two-tiered system would see families and whānau with the means to privately fund medicines for their children, having access to life-saving treatment, while those without means would miss out. As well as the possibility of opening up privately funded paediatric cancer care in Aotearoa New Zealand, some submitters said that it would mean more families and whānau had to travel overseas for treatment.

One family submission expressed relief when they found out they would not have to travel overseas, and another described how important it was to them during their child's cancer treatment that they were able to stay close to home and their extensive whānau support.

Of those who submitted on this point, many said that such a system would disproportionately impact whānau Māori and Pacific families. The National Child Cancer Network said *"[Rule 8.1b] has meant we have not ended up with a two-tier health system whereby those most marginalised and vulnerable, and those more likely to be Māori or Pasifika ethnicity don't have the same therapeutic options that the rest of the child cancer population has"*. The consensus submission from paediatric oncologists and paediatric haematologists made a similar submission saying that *"financial burden on whānau will disadvantage the already most disadvantaged"*.

Families and whānau spoke of the already *"horrific"* financial and emotional impacts of their child's cancer treatment on them, their child with cancer and their other children. These submissions explained the impacts they saw if Aotearoa New Zealand moved to a system where privately funded access to paediatric cancer medicines was better than publicly funded access.

> ❝ If [rule 8.1b] did not exist, families would be forced to decide if they could afford to save their child's life…No family should have to choose between saving their child or watching them pass, based on their financial position ❞

> ❝ …currently every single child on that cancer ward is getting access to the medications they need. I can't imagine how it would be if there was an inequality of access to medication…families [would be] separated for long periods of time if they are somehow able to fund overseas travel and treatment…far worse…is families who don't even have a chance…many [are] already barely making ends meet… [there could be] two families in the same cancer ward, one has a child on a better regime because they can pay for it…could you imagine the despair and heartbreak of being that family that couldn't afford the best and kindest medication for their child? ❞

> ❝ We were fortunate to have family to help us both in time and money…I can only imagine how stressful fundraising for your living costs would be while supporting your child…having to fundraise for treatment costs is unthinkable ❞

> ❝ [If rule 8.1b changes] we would move to Australia ❞
> (submission from a family who currently travel to Australia every three months for treatment in a clinical trial)

Submissions from families and whānau Māori

2.8.8 Most submitters said that the way to achieve equity between children and AYA with paediatric cancer and / or AYA with 'adult cancers' was to extend rule 8.1b

Many submitters said that rule 8.1b was inequitable between children and AYA with cancer, including those AYA with a paediatric cancer, those AYA with other cancers, and those AYA who have a paediatric cancer but who have matured and are therefore best treated in adult cancer units. The solutions offered to solve this inequity were primarily to ensure other populations had timely access to medicines, either through an extension of rule 8.1b or new processes to achieve the same result as rule 8.1b.

The AYA Cancer Network Aotearoa, supported by the submission from the AYA Cancer Consumer Advisory Group, submitted that continuation of rule 8.1b was essential for the treatment of paediatric cancer and that the rule was already inequitable for the AYA population. They submitted that this inequity was caused by the requirement of rule 8.1b that the child with a paediatric cancer had to be treated at one of the two specialist child cancer centres.

> ❝ The importance of keeping rule 8.1b with the wording 'people with paediatric cancers' instead of 'children with cancer' can not be overstated. It is critical to ensure equal access to potentially life-saving treatment for all people affected by these cancers, regardless of age. This will avoid age-based discrimination and the further widening of the cancer survival gap for AYAs with cancer compared to children. ❞
>
> *Submission from the AYA Cancer Consumer Advisory Group*

They submitted that nearly all AYA up to age 16 were treated in child cancer services and this may be extended to those who were 17 or 18 depending on the type of disease, patient and other factors. They said that this means that while many AYA with a paediatric cancer are treated in one of the two child cancer services, some are not, and therefore do not have access to medicines via rule 8.1b.

They submitted that approximately 20 AYA per year are diagnosed with a cancer where the expertise lies in paediatric cancer services, and approximately 38 AYA per year diagnosed with cancers that are familiar to clinicians working in both child and adult cancer services. The latter are likely to be treated in adult cancer services, which limits these AYAs access to clinical trials they are eligible for.

The AYA Cancer Network therefore submitted that *"The many compelling arguments for why rule 8.1b is so important for children with cancer equally apply for all AYAs diagnosed with a paediatric cancer"*. They said that extending the rule to AYAs *"with paediatric type cancers"* would not place pressure on the CBP, saying that in 2021 there were 15 AYAs diagnosed with a paediatric cancer, nine of which were treated in a child cancer centre and therefore already eligible under rule 8.1b if required.

For AYA with adult cancers, the AYA Cancer Network noted that they *"would also greatly benefit from a streamlined process to access evidence-based optimal cancer treatments, including clinical trials"* and said that their *"ideal is that Pharmac would consider a similar rule for AYAs who have an 'adult cancer'"*.

Other submissions on this point focused less on AYA having 'paediatric cancers', and more on the inequity between AYA with any type of cancer and children with a paediatric cancer. There were a few submissions that said rule 8.1b should be amended so that children and AYA were eligible by age (with varying submissions up to the age of 29) and not by type of cancer (i.e., the rule should facilitate the funding of all cancer medicines for those up to that age, regardless of the type of cancer). The submitters said that the reason for this was because of the same reasons children with cancer should retain access to medicines through rule 8.1b – notably their cancers are aggressive, New Zealand has societal, legal, and ethical obligations to protect children and young people, and they face the same challenges with access to suitable and effective cancer medicines. One submitter said that there should be a higher age limit for Māori and Pacific peoples.

2.8.9 Many submitters said that the way to achieve equity between children and AYA with paediatric cancer and with other rare diseases was to extend rule 8.1b

Most NGOs submitted that rule 8.1b should be extended to children and/or AYA with other rare diseases to reduce the inequity of access and outcomes between paediatric cancer and those rare diseases.

Rare Disorders New Zealand submitted that rule 8.1b should be extended to all children with rare disorders because:

a. rare disorders can have a poor prognosis and a lack of effective, known, and prescribed treatments. This means there are benefits to participating in clinical trials as part of the standard of care. This also means children with rare disorders and other chronic conditions are currently at an access and treatment disadvantage

b. children with rare disorders (noting child cancer is a rare disorder) face similar characteristics to paediatric cancer and are likely to face similar challenges accessing medicine

c. there are significant potential gains to be made by early intervention with medicines. These gains are larger than for other patient population groups, given the young age of patients and the expectation that treatment will extend survival with a good quality of life

d. their 2022 survey found that 1 in 3 people with a rare disorder are often unhappy or depressed, and there are wider economic costs of not treating rare diseases early.

To summarise, Rare Disorders New Zealand submitted that *"Rare disorders [are] either life-limiting or [the] cause of serious morbidity [and] 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"*.

Medicines New Zealand submitted similarly and stated that the current approach to funding treatment for other *"seriously debilitating and/or life-threatening conditions including rare disorders is not consistent"* with obligations under the UN Convention on the Rights of the Child.

Waitaha Canterbury, Medicines Utilisation Service in the Department of Clinical Pharmacology[11] supported the submission that a solution was needed for other rare diseases. They said that: *"A mechanism for all rare diseases… that provides timely funding assessment for patients that are not clinically exceptional is required"*.

A few submissions from families and whānau said that rule 8.1b should be extended to children with other rare diseases, particularly for paediatric populations that relied on clinical trials for treatment.

11 This submission noted that they supported the collective submission of paediatric oncologists and paediatric haematologists and that their submission was on behalf of Te Whatu Ora Waitaha health professionals, support services and the business who acknowledge they do not predominantly work with children who have cancer.

2.8.10 A few submitters were clear that paediatric cancer was different to other rare diseases and therefore rule 8.1b should not be extended

The consensus submission from paediatric oncologists and paediatric haematologists said that they did not think that rule 8.1b was inequitable for other rare diseases (in children or adults). They submitted that the rule was an *"appropriate, pragmatic and efficient funding mechanism ideally suited to the dynamic therapeutic and clinical trial environment of paediatric oncology"*.

This was supported by some submissions from family and whānau, concerned individuals, and NGOs that paediatric cancer was different to other rare diseases. This is illustrated by the following quote from a family submission:

> *"If another disease becomes the most deadly childhood condition, then that funding for that condition could be considered at that time"*

Thank you

Pharmac would like to thank everyone who contributed to the consultation on the review of rule 8.1b. We acknowledge the time people took to respond and the impact of this process on families, advocacy groups, and clinicians supporting children with cancer. As we progress with the review we will continue to keep in contact with submitters and share information with the public.

PHARMAC
TE PĀTAKA WHAIORANGA

Pharmaceutical Management Agency
Level 9, 40 Mercer Street, PO Box 10254, Wellington 6143,
New Zealand Phone: 64 4 460 4990 - www.pharmac.govt.nz
Freephone Information line (8am–5pm weekdays)
0800 66 00 50