Submission to Pharmac on the consultation around **Rule 8.1(b)** of the Pharmaceutical Schedule

From the Child Cancer Foundation

March 2023

> *I would hate for other families to have to go through more trauma than necessary because of a change in the rule that doesn't make sense. Why do anything to make that lifelong journey more fraught or more difficult or complicated?* - Leigh Honnor, Taranaki

1. Introduction

1.1 The Child Cancer Foundation (CCF or the Foundation) would like to thank Pharmac for the opportunity to provide feedback on the consultation around Rule 8.1(b) of the Pharmaceutical Schedule. The Foundation notes that the purpose of the consultation is to ascertain if changes to the rule need to occur. In the Consultation Document Pharmac asks a range of questions. CCF do not feel we are fully qualified to answer specifically questions one through seven but have attempted to do so within the context of equity and equality.

1.2 We note that the terms equity and equality and the variations inequity and inequalities are used interchangeably throughout the consultation document and associated literature. For the elimination of doubt the following definitions are used in this submission:

- Equity is the unequal distribution of resources to ensure an equal outcome.
- Equality is the same or similar distribution of resources which may or may not ensure an equal outcome.
- Inequalities are disparities between groups which may be the result of resource distribution or other factors impacting on individual groups.
- Inequities are sometimes used in the literature to describe inequalities for the purposes of this document unless quoting the literature we prefer to use the term inequalities.

1.3 The Foundation is a charitable membership organisation which provides support to families who are experiencing a child living with a cancer diagnosis. Our vision is to walk alongside and support all children and their families on their cancer journey and advocate improvements to child cancer care. In the 2021/2022-year CCF supported 367 families undertaking this journey from Te Kao in the north to Invercargill in the south, and more broadly provided peer support services to 1,300 families across the motu.

1.4 Paediatric cancer is a leading cause of death for children and adolescents globally.[1] While innovation in cancer treatments is making huge strides, generally children must wait longer to experience the same benefit newer therapies offer than their adult counterparts. The time period between first in adult studies versus first in children studies is typically six-and-a-half-years.[2] Inequality in care and access based on socioeconomic and geographic factors means survival rates for children and adolescents with cancer doesn't exceed 29% in some low and middle-income countries.[1]

[1] *World Health Organization. Childhood Cancer. December 13, 2021. https://www.who.int/fr/news-room/fact-sheets/detail/cancer-in-children*
[2] *Neel DV, Shulman DS, DuBois SG. Timing of first-in-child trials of FDA-approved oncology drugs. European Journal of Cancer. 2019 May 1; 112:49-56.*

1.5 Unlike the global situation illustrated in 1.4, paediatric oncology services in Aotearoa/New Zealand can be held up as a shining example within the wider health system of what provision of services that deliver equality of access look like. This in part, is attributable to timely access to cutting edge therapeutics and the excellent care provided by our specialist services. We have an integrated system from first diagnosis through to provision of care and access to treatments. Other disease pathways are not so fortunate. We have almost achieved equality of outcome irrespective of ethnicity, locality or several other factors such as socio-economic status. CCF believes it is important that we keep moving forward to ensure and maintain equality of access for tamariki Māori and Pacifica children.

1.6 The Foundation will also be looking for a solution that lifts outcomes for other treatment pathways. We do not want to see a solution that impacts on the success of paediatric oncology and other disorder outcomes (which is to say, equity up not equity down).

2. Recommendations

The following section provides an overview of our recommendations:

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

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

2.1.3 We further recommend that rule 8.1(b) is expanded to other conditions impacting children so that health outcomes for children overall are improved. Further we would advocate for more effort across the health and welfare (systems) to support whānau living in poverty or deprivation to further reduce inequalities between Māori and non-Māori.

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

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

3. CCF Feedback on the Discussion Document

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

3.1.1 Overall childhood cancer survival outlined in the Pharmac discussion paper *'Funding of paediatric cancer treatments in New Zealand'* indicates a 5-year survival rate of 86% across all childhood cancers. Childhood cancer incidence rates in Aotearoa /New Zealand are comparable to rates reported in Australia and from other high-income countries in Europe and North America.[3] What this figure hides are that despite the positive outcome for Aotearoa/New Zealand's children when compared globally, inequalities still exist for Māori who between 2010 and 2019 had a 5-year survival rate of 81%, seven percent below non-Māori.[4]

[3] *National Child Cancer Network. 2022. Childhood cancer incidence in Aotearoa, New Zealand 2015-2019.*

[4] *Childhood Cancer Survival in Aotearoa, New Zealand 2010 – 2019 National Child Cancer Network. 2022.*

3.2 ***Question Two: In what other clinical contexts is participation in clinical trials the 'standard of care'?***
Question Three: To what extent is access to paediatric cancer clinical trials dependent on access to medicines through rule 8.1b?
Question Four: How sensitive is this system of care to changes to rule 8.1b?

3.2.1 CCF understands that participation in clinical trials is a foundation of the overall model of care in paediatric oncology in Aotearoa/New Zealand. Continued participation in clinical trials is premised on Aotearoa/New Zealand maintaining global standards of care, which includes access to drug treatments under rule 8.1(b).

3.2.2 While questions three and four require a certain degree of conjecture, it is easy to suppose that any change to the system would negatively impact the viability of Aotearoa/New Zealand's continued participation in global clinical trials (if the standard of care where to drop below international benchmarks). We estimate that through participation in global clinical trials Aotearoa/New Zealand receives an additional 38% investment in paediatric oncology drug treatments that would otherwise be unavailable within the New Zealand health system. We additionally proffer a view that in part, some of our clinical workforces are also engaged in the system because of the opportunity to participate in global trials, which provide job enrichment.

3.2.3 Given there is little evidence (other than outcomes) to concisely address questions three and four of the consultation document in any manner other than speculative, CCF recommends that Pharmac uses its powers under 69(1)(c) of the Pae Ora (Health Futures) Act 2022 to engage "in (independent peer reviewed) research to meet the objectives set out in section 68(1)(a) (of the Act), specifically to secure for eligible people in need of pharmaceuticals, the best health outcomes that are reasonably achievable.

3.2.4 System sensitivity to changes in Rule 8.1(b) is substantial (subject to what it may be replaced by) given it is an integral part of a system that has achieved equality of access for children diagnosed with cancer. The entire system and component parts (e.g., shared care, national protocols, Specialist Hubs, Rule 8.1(b) and no private provision) work in harmony to enable world class outcomes are achieved. Disaggregating any component part will have an impact and potentially negative downstream consequences too (see below).

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

3.3.1 Rule 8.1(b) is part of a 'model of care or treatment pathway' which supports paediatric cancer patients in Aotearoa/New Zealand. Without rule 8.1(b) it is reasonable to question whether we would see health outcomes for many in this patient population being the same or similar as those in similar high-income jurisdictions.

3.3.2 Removing this option or reducing it in anyway will very likely see children in Aotearoa/New Zealand having lesser outcomes for childhood cancers than those in other high earning countries. This is not to suggest that there are no other factors in play, clearly the nationally integrated service model with two regional hubs, shared care and national protocols also plays its part in achieving these outcomes.

> *"The whole is greater than the sum of the parts."* - Aristotle

We have a range of inputs and capabilities from which has emerged a complex system which in real terms has seen near health outcome equality in paediatric oncology.

3.3.3 Even though these various individual inputs cannot be singularly denoted as the key determinant to achieving this result, the combined properties and capabilities of the larger system, achieve this positive outcome. For one part of the system to be withdrawn in whole or in part may very well create detrimental impacts or unintended consequences, such as the loss of highly sort after clinical staff who may prefer to work in a system which allows for a degree of academic engagement.

3.3.4 While CCF is concerned with whānau experiencing a cancer diagnosis in one of their tamariki, we support the view that health outcomes for children overall would be improved if the rule was extended to other paediatric diseases or conditions and more effort across the (health and welfare) systems occurred to support whānau living with a disability,in poverty or deprivation.

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

3.4.1 As noted above when viewed through a systems lens, timely access to paediatric cancer treatments is an important piece of the overall model of care. Given that the primary health goal of treating cancers in children is curative and based on effectiveness [for many patients, irrespective of demographics] of the paediatric oncology treatment pathway, the investment in timely access to pharmaceuticals is without question. We are of the view that the original rationale for Rule 8.1(b) has not changed since it was introduced, these factors are:

- the specialised nature of some treatments.
- usage differences between children and adults.
- the small number of patients each year for most indications.
- the enrolment of some patients in international clinical trials.
- some of the medicines and indications being unregistered.

3.4.2 Given five-year survival rates for paediatric cancer patients averages 86% and the fact the vast majority go on to live healthy and productive lives, the average cost of treatment of around $8000 per patient[5] would appear to be a 'value for money' investment for the New Zealand taxpayer.

3.4.3 The second part of this question is more difficult to address. It, alongside questions eleven, twelve and fifteen *[How might we address equity and fairness concerns related to paediatric cancer medicines through rule 8.1b and access to medicines for other groups? Do you consider rule 8.1b to be inequitable from the perspective of other children or those with rare disorders? Why? How might we address equity and fairness concerns related to paediatric cancer medicines through rule 8.1b and access to medicines for other groups?]* are unfortunate and rather provocative questions in our view and set-up a race to the bottom. Pharmac's remit is to lift treatment accessibility for all children rather than potentially creating conflict between different groups of people within different disease cohorts. Further we are of the view that these types of questions privilege non-Māori. Given finite resources there is always going to be a need to draw a line somewhere. Removal of 8.1(b) is likely to reintroduce inequalities into a system which has been working hard to and has almost reached a position of no inequality between Māori and non-Māori.

3.4.4 CCF does not dispute the fact that rule 8.1(b) creates inequalities of access to certain therapeutics between some children with a cancer diagnosis and those with other rare conditions, nor do we dispute that there are possible inequalities between those accessing paediatric oncology services and those accessing adult cancer services. At the same time, we would make an argument that healthcare spend generally creates inequalities between children and young people who are generally underserved (in comparison to adults), and Māori and non-Māori.

3.4.5 For example, Mills et al. conclude that *"Persistent child health inequities result in significant societal economic costs. Eliminating child health inequities, particularly in primary care access, could result in significant economic benefits for New Zealand. However, there are conceptual, ethical and methodological challenges in estimating the economic cost of child health inequities. Re-thinking of traditional economic frameworks and development of more appropriate methodologies is required."*[6] *They go on to say "child health inequities between Māori and non-Māori in New Zealand are cost saving to the health sector. However, the societal costs are significant. A conservative "base case" scenario estimate is over $NZ62 million per year, while alternative costing methods yield larger costs of nearly $NZ200 million per annum. The total cost estimate is highly sensitive to the costing method used and Value of Statistical Life applied, as the cost of potentially avoidable deaths of Māori children is the major contributor to this estimate."*

[5] *Funding of paediatric cancer treatments in New Zealand. Discussion paper on rule 8.1b of the Pharmaceutical Schedule. Pharmac 2022.*
[6] *Mills, C., Reid, P. & Vaithianathan, R. The cost of child health inequalities in Aotearoa New Zealand: a preliminary scoping study. BMC Public Health 12, 384 (2012). https://doi.org/10.1186/1471-2458-12-384*

3.5 Question Seven: Is our understanding of how rule 8.1(b) operates in practice correct? What else should we know?

3.5.1 In the consultation document Pharmac states that *"Expenditure on paediatric cancer treatments through rule 8.1b has not been a major concern in the past as the vast majority of medicines are accessed through the Pharmaceutical Schedule, as well as the relatively small overall budgetary impact on the CPB".* Given this we do not believe Pharmac has made a case for change.

3.5.2 It is difficult to make an accurate assessment of Pharmac's understanding of the way 8.1(b) operates due to the manner in which data is used and presented in the consultation document. For example, in section 3.6 of the consultation document it is noted that *"According to our annual expenditure data, the total cost of all paediatric cancer treatments used to treat people aged 25 and under in the 2020/21 financial year was approximately $5.5 million."* Using this information approximately one third of patients (assuming even distribution) were above 18 years of age and would have been seen as adults in adult services and therefore ineligible for treatments under rule 8.1(b). What this would suggest is that the figure of seven percent as the proportion of paediatric patients using the rule to access treatments is much higher and therefore the cost to paediatric patients of removing the rule is quantifiably greater.

3.5.3 Pharmac goes on to note *"we see an increasing risk with new cancer medicines, such as CAR T-cell therapy, which could cost more than $1 million to treat one person."* As a technology CAR T-cell therapy does show promise in terms of personalised treatment for cancers and at this stage in its development the cost to treat one patient is in the range of one million dollars. What is less clear however is whether this technology would be considered a pharmaceutical and therefore eligible for funding under the CPB or rule 8.1(b).

3.5.4 CCF believes the broader issue vis-à-vis technologies like CAR T-cell therapy is how it will be funded given it sits somewhere between a medical intervention, a laboratory procedure and a medicine. While pressure may eventually come on the wider health system to fund such technologies, it is not yet clear whether this would be Pharmac or the hospital system itself. An exception to the rule could be made regarding this evolving technology, to be reviewed via consensus following broader debate across the health system. But in the interests of a rational debate – we would respectfully suggest that this is. at this moment in time and into the near future a distraction from the issue at hand.

3.5.5 Finally in this section, CCF believes that the best people to make decisions on treatments are parents and their medical professionals, Rule 8.1(b) allows for this. Anecdotally it has been our experience that both clinicians and whānau are incredibly prudent and cautious when making medical decisions such as what drugs they use, how frequently and perhaps sadly, when to stop. Should there be any changes to the rule, ongoing engagement with clinicians is an imperative.

3.6 Question Eight: How much increase in the use of rule 8.1b do you think will happen as a result of the growing range of new paediatric cancer treatments?
Question Nine: Do you see the costs of paediatric cancer treatments accessed through rule 8.1b increasing significantly in the foreseeable future?
Question Ten: How could we assess what value paediatric cancer treatments provide against other medicines that could be funded with the same money?

3.6.1 CCF supports the aims and objectives of the Pae Ora (Healthy Futures) Act 2022, which at its core behoves Pharmac, alongside other health service providers to work in an integrated manner to support the health outcomes of New Zealanders. As such we believe that when assessing the value of medicines, Pharmac must not only focus on the cost of the medicine but use a values and wellbeing-based approach. A values and wellbeing-based approach supports patients but also reduces the overall cost of services health system wide. This may mean higher costs in some areas but at the same time may see reduced costs in other areas.

3.6.2 For example, as noted above child cancer treatment interventions are generally 'curative' as opposed to 'palliative'. Given the relatively young age of most patients, intervention with new medicines as soon as possible avoids premature death. This therefore means pharmaceutical intervention leads to a relatively high, quality adjusted life years (QALY) calculation for those who experience a paediatric cancer illness.

3.6.3 Aside from that which has already been discussed in this section, there is little evidence (other than outcomes) to concisely address questions eight through ten of the consultation document, in any manner other than speculative. Therefore, we once again recommend that Pharmac uses its powers under 69(1)(c) of the Pae Ora (Health Futures) Act 2022 to engage "in *(independent peer reviewed)* research to meet the objectives set out in section 68(1) (a) of the Act. To make any decisions that limit, change or eliminate rule 8.1(b) within the context of the current knowledge and the information provided would not be evidence-based and would risk decisions being made on faulty assumptions or incorrect use of data.

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

3.7.1 Please see section 3.2 of this submission.

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

3.7.2 Most dictionaries define equity as the unequal distribution of resources to achieve an equal outcome. Given a range of factors such as the aggressive nature of childhood cancers, the time sensitive nature of treatments and likely prognosis if treatment does not occur quickly verses the generally curative nature of paediatric oncology interventions, a case can be made that Rule 8.1(b) is not unequal. The model of care in New Zealand which includes access to treatments via Rule 8.1(b) has developed in such a way that inequalities between tamariki Māori and Pacifica children have been massively reduced in comparison to non-Māori (81, 83 and 88 percent respectively[7]). This therefore suggests that to achieve further gains we need to continue research on additional health system interventions and must also look outside the health system to other socio-economic determinants of health This will require additional research to answer this question in an evidence-based manner.

3.7.3 In adult services, looking at one cancer, we can see that Māori men had significantly poorer survival rates than non-Māori, when diagnosed with prostate cancer. Despite improvements in survival for all men diagnosed after 2000, the survival gap between Māori and non-Māori men has not been reduced with time.[8] While it is impossible to determine any one factor as the key to such divergent outcomes between child cancers and a fairly common adult cancer, the systems approach which rule 8.1(b) is a significant part of, clearly plays a fundamental difference in survivability and equality of outcome between the different population cohorts.

3.7.4 As noted above we believe it is unhelpful to engage in a discussion where children of differing age groups or children with other rare conditions are compared. While there are some significant differences between paediatric cancers and some other rare conditions CCF strongly believes that the overall model within paediatric oncology demonstrates what success looks like in treating other diseases/conditions in children. As such we would urge Pharmac to be looking at replicating its contribution to this model of care and not withdrawing it altogether.

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

3.8.1 Rule 8.1(b) removes a layer of inequality as it is presently designed. The financial cost to access paediatric cancer medicines is removed. Without the rule, access to some medicines would be restricted to those at the top end of the socio-economic scale or those who for whatever reason, are able to mobilise their communities to fundraise on their behalf. Families in the lower socio-economic cohort, of which Māori and Pacifica are over-represented generally, then face major barriers to access.

3.8.2 The publicly funded paediatric oncology system without barriers to funding for drug treatments minimises poor health outcomes for tamariki Māori and Pacific children with cancer. 8.1(b) can be viewed as an important tool to reducing structural or institutional racism and the negative outcomes that evolve from these systemic issues. CCF believes the Human Rights Commission

[7] *Childhood Cancer Survival in Aotearoa, New Zealand 2010 – 2019. NCCN 2022*

[8] *Survival disparities between Māori and non-Māori men with prostate cancer in New Zealand, Obertová Z, Scott N, Brown C, Stewart A, Lawrenson R.BJU Int. 2015 Apr;115 Suppl 5:24-30. doi: 10.1111/bju.12900.*

opinion was designed to challenge Aotearoa/New Zealand to lift access to other groups not by reducing access and funding benefits to one cohort (children with cancer) at the expense of a portion of that cohort. (Māori and Pacifica children with cancer).

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

3.9.1 Please see section 3.2, 3.7 and 3.8 of this submission.

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

3.10.1 There is an assumption in this question that Rule 8.1(b) does not address equity and fairness when in fact it allows for equal access to a high-quality standard of care and access to drug treatments irrespective of ethnicity, gender, cancer diagnosis, or postcode. The real question that needs to be asked is where in a system of finite resources do we wish those inequalities to exist? Given our failure as a society to invest in children's health at the same rate as adult's, years of accumulated under-investment have created a situation where this failure is magnified. Rather than further disadvantaging children with cancer, we urge Pharmac to look to expand the criteria of 8.1(b) to include rare disorders and chronic illness. Children and adolescents are one of the most underserved populations in Aotearoa/New Zealand, all children deserve to receive the medicines to support their health and wellbeing in the same way we invest in adults.

4. Concluding Comments

4.1.1 The outcome of any review of Rule 8.1(b) which does not result in the status quo will be the largest single impactor on the paediatric oncology treatment eco-system in a decade. As noted above the real question that needs to be asked is where in a system of finite resources do we wish those inequalities to exist? It would seem, that from a paediatric oncology perspective those within the sector would generally make the argument that if it isn't broken there is no need change it.

4.1.2 At the same time, we acknowledge that from an organisational perspective, it is difficult for Pharmac to manage a budget when it doesn't have control of the outputs. Notwithstanding this the consultation document makes assumptions that are both uncomfortable and erroneous. That is the motivations of clinicians and parents who in our view, are the best decision makers and perhaps Pharmac's best gatekeepers when it comes to pharmaceutical use. We make this observation based on our experience of both clinicians and parents, who are placed in the unenviable position of determining, almost daily, when to treat, what to treat with, and most difficult of all when to stop treatment in the best interests of the child.

4.1.3 A cancer diagnosis is an incredibly traumatic time for anyone, adult or child. What is perhaps more challenging for children and their whānau is the possible lost potential a cancer diagnosis can be. The challenge for those of us who work in the sector more broadly is to provide as much support as we possibly can so the cancer journey is a easy as it can be. And that is where the consultation document does not achieve this aspiration. We submit with respect, that the document fails to make a case for commencing a review into rule 8.1(b), and therefore such a review should not take place.

Dennis Turton

Board Chair | Heamana Poari ā-Motu
Child Cancer Foundation

Monica Briggs

Chief Executive | Tumu Whakarae
Child Cancer Foundation

> *"I really feel for families moving forward who find out that their child has cancer and the potential hardship that they may face on their journey with treatment potentially not being subsidised or accessible. I feel really pouri – really sad – for all those whānau. I don't know how people will be able to cope with additional layers put on top of them."*
> - Ori Nolan-Edwards, Palmerston North