Record of the Cancer Treatments Advisory Committee
Meeting held on 26 May 2021

1. The inconsistencies associated with the current arrangement for paediatric cancer treatment funding

Attendees
Present from the Cancer Treatments Subcommittee:
Allanah Kilfoyle
Chris Frampton
Lochie Teague
Marius Rademaker
Peter Ganly
Richard Isaacs
Tim Hawkins

PHARMAC staff
Alison Hill
Beth Caudwell
Caroline De Luca
Geraldine MacGibbon
Laura Baker
Logan Heyes
Rachel Read
Scott Metcalfe
Simon Mitchell

Application

1.1. The Subcommittee noted a briefing paper prepared by PHARMAC staff to discuss the current arrangement for paediatric cancer treatments and a potential approach to address inconsistencies.

Discussion

1.2. Members noted that current funding for paediatric cancer treatments, for the treatment of cancer, not available via the Schedule occurs via a notification of use to PHARMAC. The Members noted that this means that there is no delay in access to treatment when it is needed and that this is inconsistent with how access to other medicines on the schedule are funded. Members noted that paediatric cancers tend to be very aggressive and that most treatment is provided with curative intent. Members noted that the vast majority of treatments used by paediatric cancer patients are already available on the schedule and it is only a minority that receive medicines not available to other patients via this notification process.

1.3. Members noted that some treatments are made available free of charge, either due to use as part of a clinical trial or for certain unapproved medicines that the supplier provides as free stock. In general, for clinical trials, it is the standard of care that is publicly funded through rule 8.1b of the Pharmaceutical Schedule. Members noted that approximately one third of paediatric cancer patients receive treatment as part of a clinical trial and funded access is required for treatments used as standard of care (non-investigational product), which are often multi drug complex protocols that include drugs not listed on the Pharmaceutical Schedule.

1.4. Members noted that the status quo has served this patient group very well and has provided good outcomes compared to other OECD countries. In addition, these good outcomes are not influenced by domicile or ethnicity. Members however considered that these equity benefits (location and ethnicity) were primarily driven by the service and less so access to medicines.

1.5. Members noted that there were issues with other patient groups and medicines considered for funding as per PHARMAC process. Members considered that the lack of rules currently is difficult, that age is important but not explicit, and ultimately trying to make paediatric oncology fit into the adult process would be very challenging. Members considered that there was a need for a more universal process required for all cancer medicines, given the difficulties that have arisen with the involvement of the adult oncology services in New Zealand in adult oncology trials.

1.6. Members noted that AYA patients in the paediatric service receive access to treatment in the same way. However, patients who are not treated within the paediatric service for cancers more common in paediatric patients would not receive access to treatment in the same way and would be treated by the adult oncology service. Members considered that this was an issue that needed to be explored and that people outside the paediatric age group, requiring treatment with medicines for cancers predominantly seen in paediatric patients should receive the same access regardless of the type of service. Members noted that the AYA age group has been increasing in recent history and now includes patient up to 30 years of age. Members considered that there are certain cancers with a bimodal age distribution, so in order to address the equity concerns, it may be necessary to limit Special Authority by indication rather than age group.

1.7. Members noted that there was a Human Rights complaint received by PHARMAC, however considered that there were important and distinct differences between different diseases. Members noted that paediatric cancer treatments are provided with curative intent, have afforded patients very good outcomes in the past and the fiscal risk is small compared to other treatments for other patient groups where the treatments are not curative and are ongoing.

1.8. Members considered that it was important to develop a framework to define availability and access to treatment for paediatric cancer patients, as this is a part of the concern that has been raised by other groups. Members considered that the current arrangement was ideal for this patient group, but that there was no governance over the potential cost of treatment for this patient group and it is therefore not sustainable. Members considered that it was important to preserve good outcomes that have been afforded noting the public support for this patient group. Members considered that the process for funding needed to be transparent and aligned to the evidence base.

1.9. Members noted that it can be difficult to obtain robust evidence for this patient group and that it would be difficult to require Medsafe approval prior to listing on the schedule for medicines used in this context.

1.10. Members noted that a new treatment, which requires the same notification process for funded access does involve peer review by relevant members of the paediatric service. Members noted that in some cases, for various reasons, the treatment is not put forth after this review. Members also noted that a similar review process occurs in adult oncology, when considering a research project or exceptional circumstances application.

1.11. Members considered that there were new treatments coming through the pipeline at substantial cost, that should be reviewed against the Factors for Consideration. However, that it was important that these reviews were timely and that there was sufficient and varied expertise when reviewing these applications.

1.12. Members considered that for the medicines already listed on the schedule, a transition would be relatively simple. However, for medicines that are not listed on the schedule but currently being used, there is a difference between grandparenting patients receiving medicines and grandparenting the medicine itself. Members considered that grandparenting the medicine was a useful catch all as it would contribute well to maintaining the status quo. However, for future applications, there is a need for timely review and access to treatment. Members considered that in most cases this would

require a review rather than a formal funding application. Members considered that it would be important to review all proposed Special Authority criteria changes prior to implementation.

1.13. Members considered that given the small patient group requiring new treatments, a bespoke process analogous to the exceptional circumstances process would be reasonable to address the timeliness concerns. However, members considered that there would come a time where a more formal funding evaluation would be required, if the patient group was larger than previously thought, or if the medicine was needed for the wider group up front as part of the trial. Members considered that it would be difficult to define what would warrant a wider submission and what would be reasonable to request access for via a bespoke exceptional circumstances application.

1.14. Members considered that it would be important to have a multidisciplinary panel specifically available for review of applications, and while it was important to have paediatric oncology/haematology expertise involved, there was also a need for oversight from other relevant clinicians (e.g. paediatricians, adult oncologists etc.). Members considered that documentation regarding the medical decision making within the paediatric service should be provided to PHARMAC for review by the relevant panel.

1.15. In addition to the paediatric oncologists/haematologists in Auckland and Christchurch, members identified who to engage with to identify the most meaningful stakeholders to engage with from a Māori and equity perspective. In addition to this, members considered that it would be useful to engage with patient advocacy groups, such as Child Cancer Foundation, Leukaemia and Blood Foundation, Canteen and Cancer Society and The Paediatric Society of New Zealand. Members also considered that it may be important to engage with relevant rare disorders stakeholders and the rare disorders Subcommittee. Members considered that it would be beneficial to engage with the Consumer Advisory Committee, as well as the Cancer Control Agency and the relevant working groups.

1.16. Members considered that the current system works well and is highly successful, and therefore any new process would ideally not deter from or impact on the outcomes achieved. Members considered that access to treatments for a short period of time, in a research setting is not something that has been successful previously for other patient groups and there was a risk of a similar outcome in this patient group that historically has very good outcomes.

PHARMAC
TE PĀTAKA WHAIORANGA

MEMORANDUM FOR CONSUMER ADVISORY COMMITTEE
12 AUGUST 2022

To:	Consumer Advisory Committee members
From:	Stephen Tat, Senior Policy Advisor Alex Maplesden, Senior Communications Advisor – Strategic Allanah Andrews, Manager - Policy and Government Services
Date:	August 2022

Discussion paper on paediatric oncology and haematology treatments

Purpose

In April 2022, we presented you a paper that proposed a two-stage consultation on changes to rule 8.1b of the Pharmaceutical Schedule. We sought your feedback on what questions we should be asking in a discussion document as part of the first stage of this consultation.

A working draft of the discussion paper is attached for your consideration.

Background information

Rule 8.1b of the Pharmaceutical Schedule

The Pharmaceutical Schedule provides a list of all the medicines that are publicly funded in New Zealand. It also includes a section on the general rules and restrictions that apply to subsidies for funded medicines.

Rule 8.1b of the Pharmaceutical Schedule enables exceptions to the usual process for accessing pharmaceuticals, allowing public hospitals to give (and be eligible to receive a subsidy for) any pharmaceutical for use within a paediatric oncology/haematology service for the treatment of cancer. It means that we do not require pharmaceuticals used under this pathway to undergo the same decision-making processes as is required for normal listings, or for applications under the Exceptional Circumstances Framework.

Revisiting our review of the rule

We began the review of rule 8.1b review because of the need to ensure all our policies are aligned with our statutory objective of achieving the best possible health outcomes for eligible people requiring pharmaceutical treatment.

The rule raises concerns about the equitable treatment of paediatric cancer patients compared to other patient groups due to the inconsistent funding pathways available, and that the current funding approach for these treatments may not be sustainable, with expensive new cancer treatments such as CAR T-cell therapy on the horizon.

The review therefore intends to assess whether rule 8.1b:

- effectively uses available resources
- ensures pharmaceutical expenditure is sustainable
- contributes to equitable health outcomes.

Developing a discussion paper

At the April meeting, we explained we would take a two-stage approach to public consultation, beginning with a discussion paper to help us understand the rule, and then an options paper that identifies how we would like to move forward.

While you were generally supportive of the rule as it was currently and emphasised the importance of prioritising children, you noted that we should focus on paediatric oncologists' opinions and knowledge given this was their area of expertise.

The discussion document provides clear context to rule 8.1b and why we are reviewing it without making any conclusions about future options. Several discussion questions in the paper highlight areas where we need more information or insight into. They are broadly organised into three themes:

- How effective is the current policy in terms of achieving the best health outcomes?
- Does the current policy support efficient and sustainable use of available resources?
- Does the current policy support equity?

The feedback we receive from this discussion paper will shape the options paper later in the year. We want feedback from a range of stakeholders, clinical and consumer, so it is critical the discussion paper is reader-friendly and well-structured to elicit carefully considered feedback.

Questions for your consideration

We encourage you to read the discussion paper and consider the following questions for discussion at the CAC meeting:

- Do you think the discussion paper is in plain language and suitable for our intended audience?
- Does the discussion paper effectively communicate our focus on equity and te Tiriti o Waitangi?
- How do you think people receiving paediatric oncology/haematology care will feel when reading this paper?
- Are there any other questions you think should be included within the discussion paper?
- Do you have any general feedback or other suggestions?

Minutes of the PHARMAC Consumer Advisory Committee (CAC) Meeting Friday 12 August 2022

The meeting was held via zoom from 10.00 am.

Present:
Lisa Lawrence (Chair)
Hazel Heal
Janfrie Wakim
Mary Schnackenberg
Nele Kalolo
Robyn Manuel
Sione Vaka
Vivien Verheijen

Pharmac staff in attendance:
Jannel Fisher (Manager, Implementation, Communications and External Relations)
Mako Osborne (Graduate Implementation Advisor)
Peter Alsop (Director Engagement and Implementation)

For relevant items:
Alex Maplesden (Senior Communications Advisor)
Stephen Tat (Senior Policy Advisor)

1. Karakia and welcome

The meeting was opened with a karakia.

Out of scope

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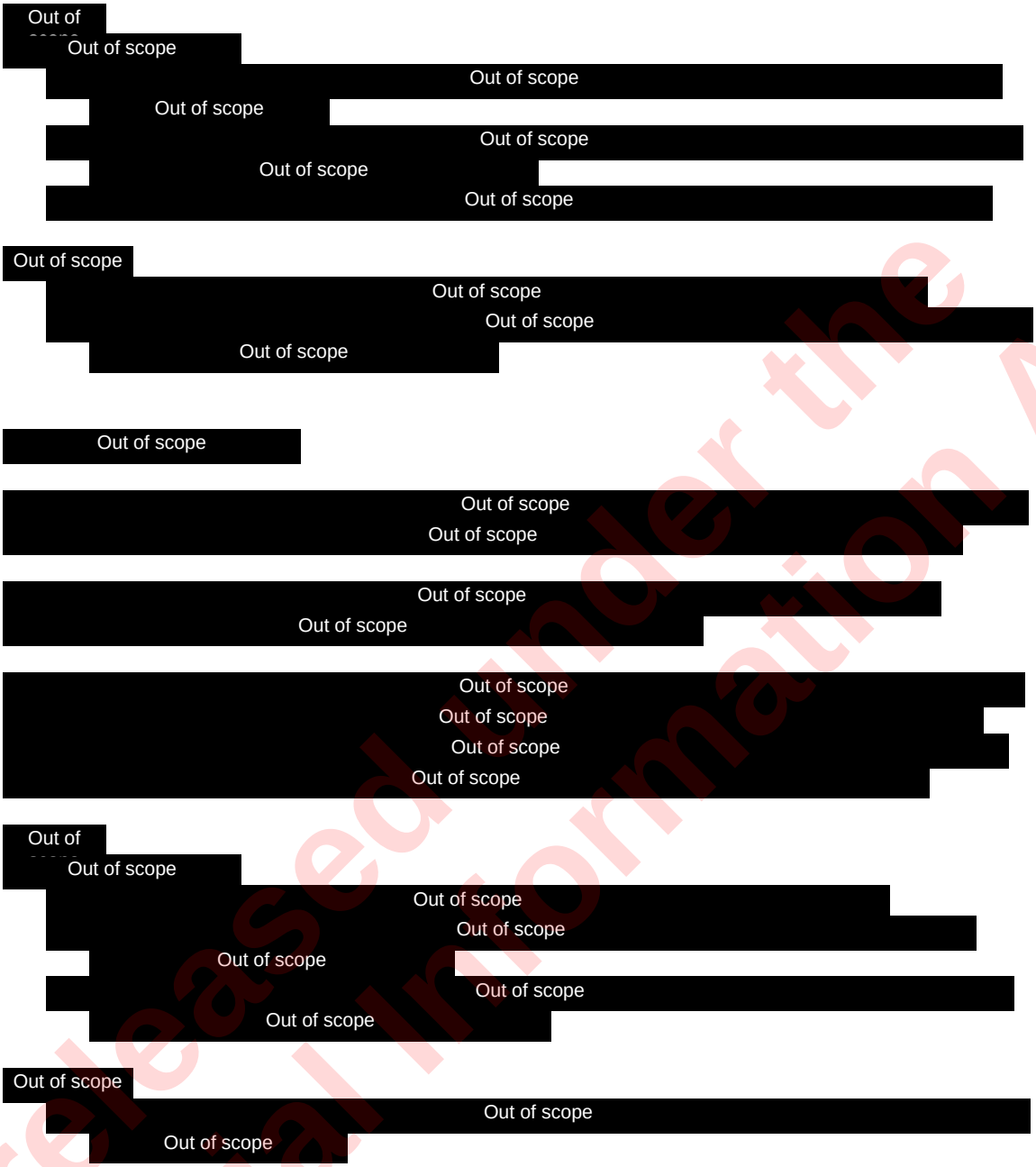

8. Paediatric oncology treatments

Senior Communications Advisor, Strategic and Senior Policy Advisor gave a summary of the draft discussion paper for paediatric oncology treatments.

Members discussed taking different approaches to ensure a variety of audiences can fully comprehend and interact with the content. Members suggested ways that different demographics could be targeted, which included complimentary derivative products, a more succinct summary and the use of visuals and spoken language.

Members noted that this is a complex discussion, and it is important to demonstrate the context of equity in this situation.

Members noted that those with lived experiences will be nervous about this review and encouraged Pharmac to reassure affected patients and whanau that there will be no change to their care.

ACTIONS:

1) *Members to email any further comments.*

Out of scope

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