PTAC Pharmacology and Therapeutics Advisory Committee

Objective advice to PHARMAC

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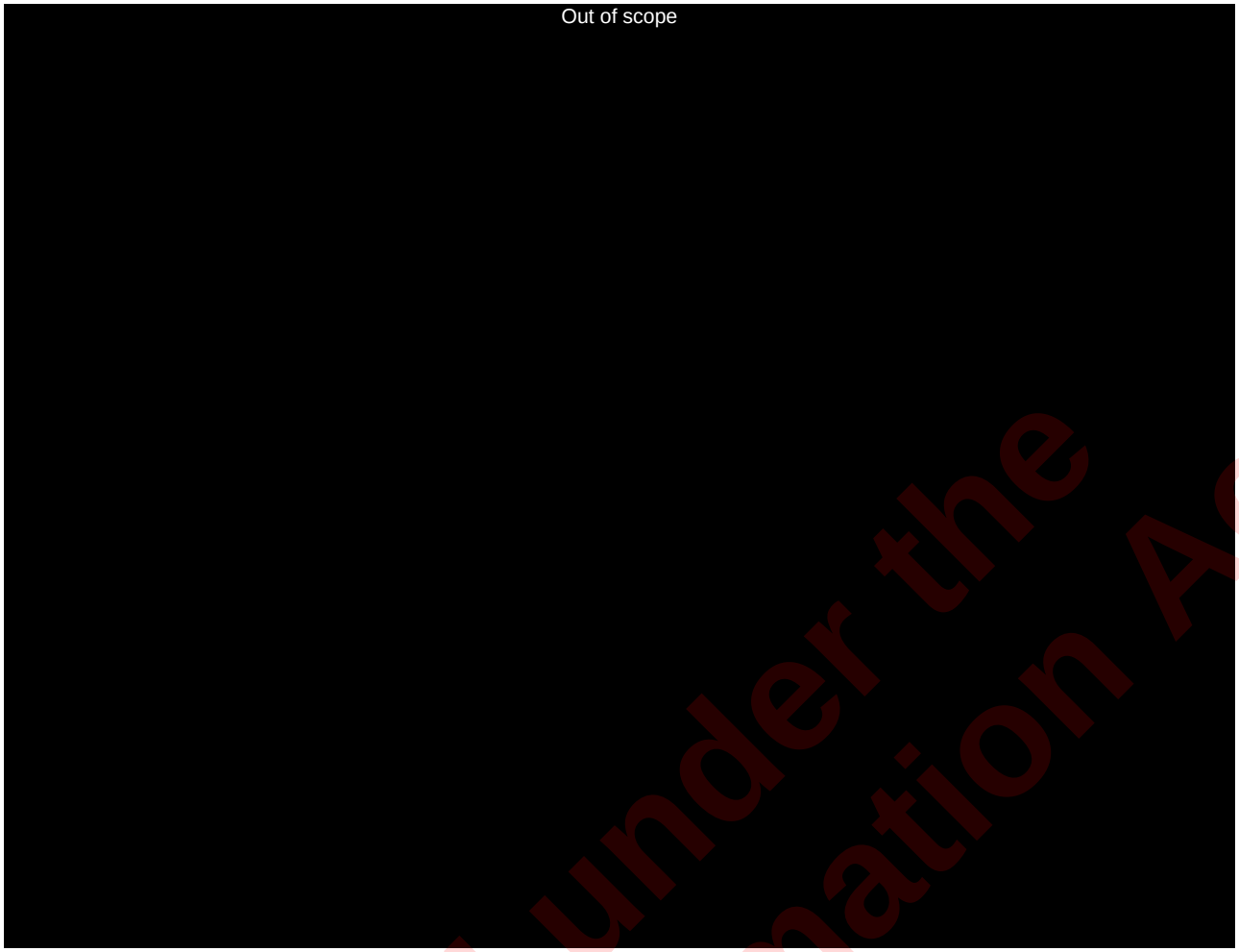
Record of the Pharmacology and Therapeutics Advisory Committee Meeting

Held on 19 and 20 August 2021

This meeting was held via videoconference

Chair



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

Interests

11.1. The Committee reported no conflicts of interest with regard to this agenda item.

11.2. The Subcommittee noted a briefing paper prepared by Pharmac staff to discuss the current arrangement for paediatric cancer treatments and the complexities regarding any change to the current arrangement.

Discussion

11.3. The Committee noted that current funding for paediatric cancer treatments, for the treatment of cancer, not available via the Pharmaceutical Schedule occurs via a notification of medicine use to Pharmac through the use of Rule 8.1b of the Schedule.

11.4. The Committee noted that there are 15 paediatric oncologists and/or haematologists treating approximately 150 new paediatric cancer patients each year in New Zealand. The Committee noted that nearly all of this treatment is delivered in the public health sector. The Committee noted that children are treated according to formal research protocols 35-40% of the time and that a number of these protocols contain medicines that are not approved for funding (outside of Rule 8.1b). The Committee noted that the current access pathway for this patient group involves no substantial delays in being able to prescribe oncology drugs for children, including those medicines that are expensive. The Committee however noted that it was important that all trials of novel protocols would need to be in line with the exemptions for clinical trial provisions of Section 30 of the Medicines Act.

11.5. The Committee noted that some treatments are made available free of charge, either due to use as part of a clinical trial or for particular unapproved medicines that the supplier provides as free stock. The Committee noted that in general, for clinical trials, it is the standard of care component in the oncology clinical trial that is publicly funded through rule 8.1b of the Pharmaceutical Schedule if necessary. The Committee noted that presently, there are many issues with the presence of, or rescindment of rule 8.1b that were not as evident when Pharmac was established, or when Pharmac took over the cancer medicines budget from DHBs.

11.6. The Committee considered that the status quo funding arrangement has served this patient group very well and has provided good outcomes compared with other OECD countries. In addition, these good outcomes are not influenced by DHB of domicile or ethnicity. The Committee considered that rescinding rule 8.1b would drive inequities, as it would be more reflective of the less accessible status quo funding arrangements for other patient groups.

11.7. The Committee considered that to date, the total drug budget for paediatric oncology is well contained, but new technologies pose a significant financial risk. The Committee understood that paediatric oncologists/haematologists expect that new technologies, such as CAR-T cell therapy would be 'grandparented' within current paediatric cancer treatment funding arrangements if rule 8.1b of the Pharmaceutical Schedule were rescinded.

11.8. The Committee noted that there were many concerns for paediatric oncologists/haematologists regarding any changes to the current arrangement.

11.8.1. The Committee noted that clinicians, patients and whānau would find it difficult if access to medicines considered to be beneficial (according to clinical consensus) was curtailed.

11.8.2. The Committee noted that the primary concern was in relation to timeliness, noting that a 'high' priority clinical advice recommendation for a medicine could still result in significant delays in funding. The Committee noted that time delays of this magnitude would be seen as serious impediments to the maintenance of current childhood cancer outcomes.

11.8.3. The Committee noted that adolescent and young adults with cancer are treated using the access supplied via part 8.1b if treated within the paediatric cancer service.

11.8.4. The Committee noted that whilst this is not a large group of patients, it is a patient group vulnerable to a change in drug availability.

11.8.5. The Committee noted that many paediatric clinical trials involve surrogate outcomes and frequent revisions to the trial protocols and that definitive clinical end-points, such as overall survival differences, often emerge well after the investigators have moved on to a new protocol. The Committee noted that by virtue of the way these patients are treated, there may be insufficient evidence using current approval mechanisms to support a strong recommendation for funding of these cancer drugs. The Committee also noted that once a submission for funding on the Pharmaceutical Schedule is made, then access via NPPA would not be possible.

11.8.6. The Committee noted that for a number of patients, if research protocols were unavailable because an unfunded drug was a component of the comparative 'standard of care', this could result in the use of the Ministry of Health's (limited) high-cost treatment pool for funding.

11.8.7. The Committee noted that access to treatments for a short period of time, in a research setting has not been successful for other patient groups previously. The

Committee noted that access to such trials in adult oncology is difficult, in many instances due to the lack of funding of treatments not funded as part of the trial.

11.9. The Committee noted that they potential solutions could be to 'grandparent' all current paediatric oncology drugs, including CAR T-cell therapy, and rescind Schedule rule 8.1b but retain a permissive NPPA process for paediatric oncology drugs. The Committee noted that alternatively, it could be appropriate to retain the current processes but move to a fixed budget, to alleviate concerns about exponential growth in approvals and costs. The Committee however noted that neither solution for paediatric oncology patients would be equitable compared with current arrangements for other patient groups.

11.10. The Committee noted internationally a study that assessed the use of a fixed budget for cancer in England and Wales (the NHS's Cancer Drugs Fund), which indicated that much of the fixed budget is spent on drugs deemed by NICE to be not cost-effective (Chamberlain et al. British J Cancer. 2014.111:1693-702). The Committee considered that ring-fenced funding is an issue and the evidence from overseas indicates that it would not be an effective means of managing a fixed budget. The Committee also considered that an outside-of-budget appropriation, much like what occurred with the recent funding of cost-effective but high budget cost new hepatitis C treatments, could be necessary to resolve this.

11.11. The Committee noted that rescinding rule 8.1b would be difficult and considered that the risks of doing so without a mitigation strategy would be high (poorer health outcomes for children; concerns raised by the stakeholders such as the news media, Human Rights Commission, Children's Commissioner, Health and Disability Commission with loss of public confidence and health sector expectations). The Committee noted that this has highlighted the already apparent bottleneck in drug funding in New Zealand, specifically the length of time that medicines spend on Pharmac's prioritisation list.

11.12. The Committee considered that this was an extremely complicated issue and that a formal review of this would require significant engagement with clinical and other experts. In addition, the Committee considered that it would be important to engage with consumers, whānau and communities about this issue in order to achieve the best, most equitable outcomes. The Committee considered that it would be important for the Government's independent review of Pharmac to review this particular area during its review of Pharmac and Pharmac processes. The Committee noted the significant service component associated with the treatment of this patient group and considered that it would be useful for Pharmac to involve Te Aho o Te Kahu (The Cancer Control Agency) in these discussions.

PHARMAC
TE PĀTAKA WHAIORANGA

MEMORANDUM FOR CONSUMER ADVISORY COMMITTEE MEETING 8 APRIL 2022

To: Consumer Advisory Committee members

From: Manager, Policy and Government Services

Date: April 2022

Review of rule 8.1b of the Pharmaceutical Schedule

The purpose of this paper is to:

- outline the proposed consultation approach for the review of rule 8.1b; and
- seek member's feedback on what questions we should be asking in our discussion document on the review of rule 8.1b of the Pharmaceutical Schedule

Background information

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.

Under rule 8.1b of the Pharmaceutical Schedule, District Health Board Hospitals (DHBs) may give (and will be eligible to receive a subsidy for) any pharmaceutical use within a paediatric (ages 0-14 years) oncology/haematology service for the treatment of cancer. Pharmac does 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.

This approach is inconsistent with how other treatments are funded through the Combined Pharmaceutical Budget (CPB) and facilitates potentially inequitable access to medicines. Given that DHBs are able to prescribe these medicines without Pharmac involvement, these paediatric pharmaceutical cancer treatments (PCTs) have not been assessed using Pharmac's Factors for Consideration or prioritised against all other medicines in the Options for Investment list.

There are no comparable pathways in place for other health conditions for paediatric, or adult populations.

The decision to undertake a review of rule 8.1b was triggered by several factors, including:

- questions have been raised regarding the equitable treatment of paediatric cancer patients compared to other patient groups due to the inconsistent funding pathways available
- concern that the current funding approach for PCTs may not be sustainable, with expensive new cancer treatments such as CAR T-cell therapy on the horizon
- Pharmac had always planned to revisit this mechanism for funding, which was not considered possible when Pharmac assumed the task of funding all cancer

treatments in 2005. This was due to complexities in relation to the treatment of paediatric cancer patients, including; the specialised nature of some of these treatments, often used differently in children than in adults; the small number of patients requiring treatment each year for most indications; and that many of these treatments and indications were not approved, or would not be likely to be approved, for use by Medsafe or other international regulatory authorities.

In public statements in response to the media interest (following a complaint made to the Human Rights Commission) Pharmac stated the following principles will guide the review:

- ensuring that all treatments currently used continue to be available to current and future patients
- ensuring that new paediatric cancer treatments are assessed in a way that is consistent with Pharmac's decision-making processes for other treatments
- ensuring that prescribers can continue to operate as similarly as possible to how they do now.

These principles pre-empt certain outcomes, which we have not yet done the analysis to support. However, given the public nature of the statements, they will remain important as we progress the review and will be an important consideration when we implement changes to the status quo (if any).

Consultation to date

In addition to internal engagement across Pharmac, we have engaged with several groups to date, including: PTAC; Pharmac's Cancer Treatments Advisory Committee; Te Aho o Te Kahu (Cancer Control Agency); Paediatric oncologists and haematologists in Auckland and Christchurch, including representatives of the National Child Cancer Network (NCCN) and Adolescent and Young Adult (AYA) Cancer Network; and Dr George Laking – an oncologist with expertise in Māori health and Co-Medical Director of the Cancer Society of New Zealand.

Annex 1 provides a summary of the feedback received to date as well as data on funding of paediatric PCTs.

Public consultation approach

Pharmac will be running a two-stage public consultation process involving:

a. First, the release of a discussion document which canvasses and invites feedback on the problem definition, objectives, and potential options (including the status quo) for reform of rule 8.1b. The outcomes of this first stage will include establishing the scope of any review and the timeframes.
b. Second, the release of an options paper which identifies the preferred Pharmac option, including implementation considerations and invites feedback on it.

We aim to have both stages completed by the end of 2022.

Questions for consideration

1. Based on information available, do you think that having a bespoke funding mechanism for paediatric PCTs is justifiable? E.g. compared to other paediatric conditions, or adolescent/young adult or adult cancer patients?

a. What additional information (e.g. data, professional opinions, analysis) would you need to fully consider this question?

2. From a consumer perspective, what are the key questions you would want answered in this review to feel comfortable about the outcome?

3. In your view, what would good consumer engagement look like for this review?

Annex 1: What we've learned so far

The following provides a summary of feedback received to date from stakeholders.

The current system promotes consistency of care and equity

- Paediatric cancer patients' outcomes are equitable for different paediatric patient groups (e.g. by ethnicity, DHB domicile or socio-economic status), representing a rare equitable outcome in New Zealand's health system. Absolute outcomes in terms of five-year survival rates for paediatric cancer patients compare well to the UK and Australia – approximately 85%.

- Treatment and care are centralised in Auckland (Starship Blood and Cancer Centre) and Christchurch (Children's Haematology Oncology Centre (CHOC)), which promotes consistency of care. Patients are referred to their regional cancer treatment centre when it is safe to do so.

Clinical trials are essential for effective treatment and access to medicines via Rule 8.1b underpins this

- Paediatric oncologists have told us that they couldn't practice with access to, or participation in a clinical trial network. Clinical trials are the standard of care for the treatment of this patient group.

- It would be difficult to exclude the use of medicines in a paediatric oncology on the basis of a lack of published evidence. Paediatric oncology trials are designed to build off each other and allow clinicians to exchange knowledge. The NCCN considered that it would be difficult to tease out exactly which components (toxicity, treatment times or outcomes etc.) of a trial benefit patients.

- The trial rate is around 30% in paediatric oncology service. These trials enable paediatric oncologists to access diagnostics unavailable in New Zealand as well as access to detailed treatment protocols, which are frequently changed and updated, which enable the effective treatment of patients. Dr Laking noted that access to clinical trials is more important than the medicines patients are being treated with as it is the treatments that enable the model of care, which in turn enable the outcomes.

- Rule 8.1b enables paediatric oncologists access to clinical trials through facilitating access to medicines that they would otherwise not be able to. For example, without Rule 8.1b, paediatric oncologists would not have been able to access clinical trials for blinatumomab and now inotuzumab. In addition, through participation in clinical trials, free medicines have been accessed for patients.

- Participation in clinical trials means that New Zealand-based paediatric oncologists are required to undergo external review of their clinical activity. This is beneficial because it ensures they are up-to-date and aligned with clinical best practice.

There are some criticisms of where the line is drawn

- Dr George Laking noted that current access arrangement for the AYA group are inequitable and considers an age demarcation to be arbitrary. Often cancers found in children and AYA population groups are biologically equivalent.

- Biology should be used a principle of differentiation. The NCCN noted that most patients aged 15-19 are already treated in paediatric units. It was also noted that many people in the AYA group have cancers better treated in the adult service, but many would be better off being treated in the paediatric service.

- In addition, part of the complaint to the Human Rights Commission was that Rule 8.1b did not allow funding for paediatric non-cancer treatments, such as for Spinal Muscular Atrophy.

The treatment model for paediatric patients would not work in adult oncology

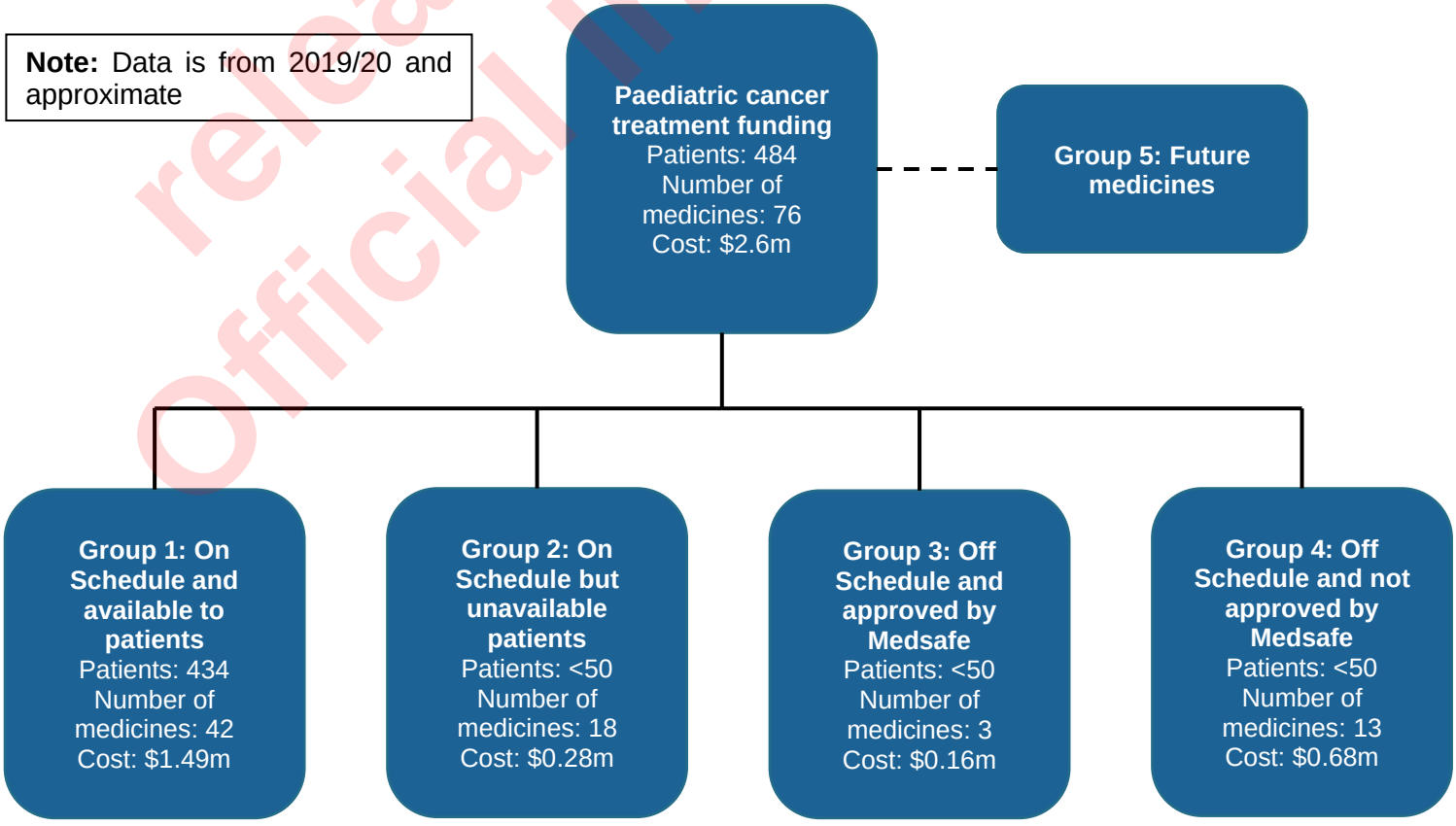
- Adult oncology considers greater number of patients. There is a lack of hard data to support proposals in paediatric oncology, more diversity of diagnosis and complexity of treatment for small numbers of patients – almost like each patient has their own disease with variations in disease morphology.

- Paediatric cancer care occurs on a collaborative, research-based model (e.g. clinical trials).

- It was also noted that current Pharmac frameworks for analysis of new medicines is based around established disease in large populations, to which this patient group is not truly applicable.

Medicines that are funded for paediatric cancer patients can be divided into four groups, represented in the diagram below (Figure 1). Additionally, there is a fifth group represented in this diagram that will require careful consideration given the expected rising costs of new paediatric cancer medicines.

Figure 1. Medicines funded for paediatric cancer treatment



Medicines in Group 1 are already listed on the Schedule and are available to paediatric cancer patients. Any change to Rule 8.1b will not affect existing patients prescribed these medicines, or future patients that may be prescribed these medicines (assuming medicines or their equivalent remain on the Schedule). Groups 2-4 of medicines are currently accessed via Rule 8.1b.

Minutes of the PHARMAC Consumer Advisory Committee (CAC) meeting Friday 08 April 2022

The meeting was held via zoom from 9.30 am.

Present:

Lisa Lawrence	Chair
Leslie Robinson	
Hazel Heal	
Nele Kalolo	
Robyn Manuel	
Sione Vaka	
Mary Schnackenberg	
Vivien Verheijen	
Janfrie Wakim	

Apologies:

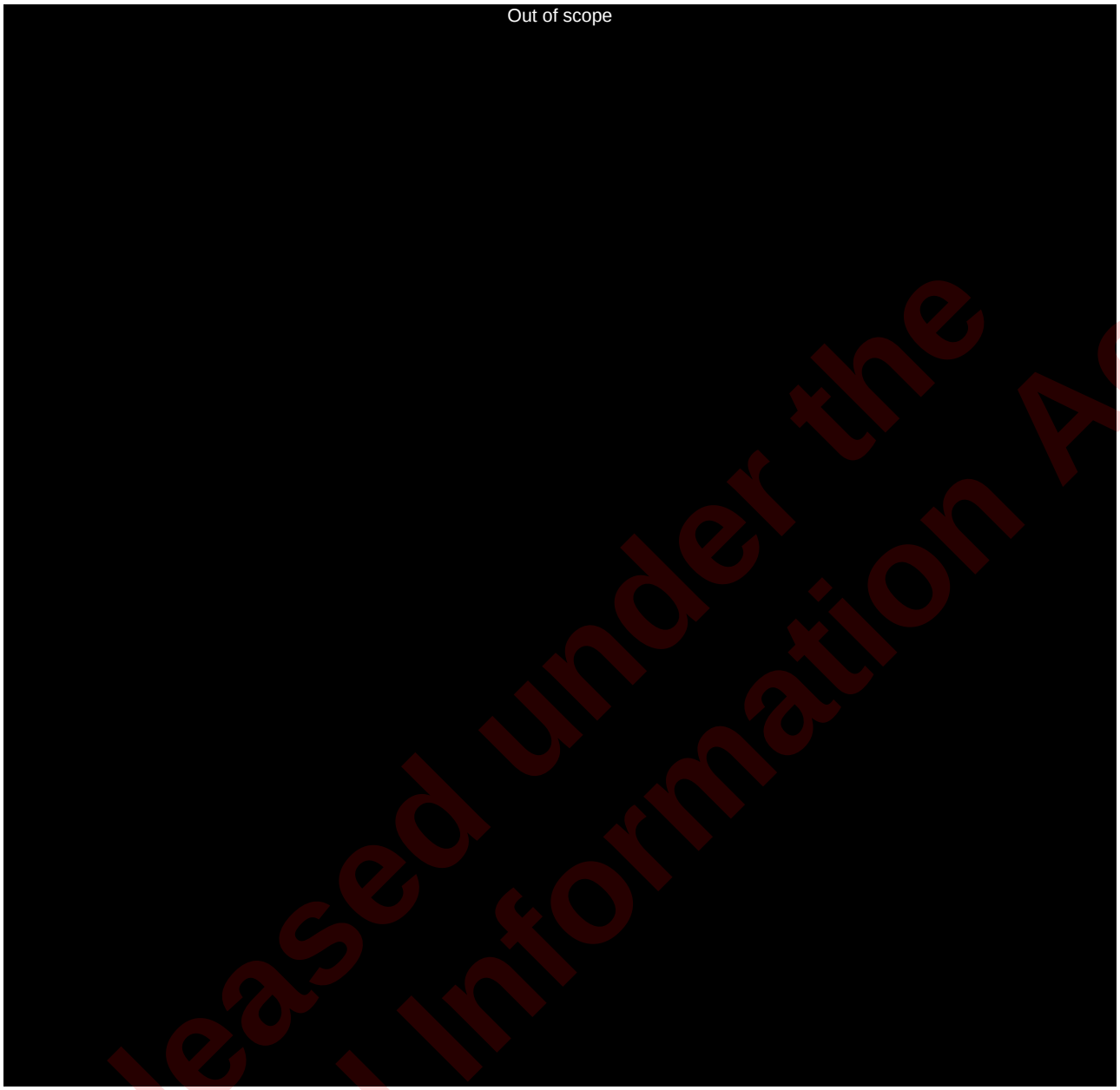
PHARMAC staff in attendance:

Peter Alsop (Director Engagement and Implementation), Janet Mackay (Manager Implementation Programmes), Mako Osborne (Graduate Implementation Advisor)

For relevant items: Megan Nagel (Senior Implementation Lead), Danae Staples-Moon (Manager, Device Strategy and Development), Allanah Andrews (Manager, Policy and Government Services), Stephen Tat (Senior Policy Advisor), Angela Cathro (Kaiwhakahaere Te Whaioranga - Manager Te Whaioranga), Jannel Fisher (Manager, Communications and External Relations)

Out of scope

Out of scope



8. Paediatric Oncology Treatments

Pharmac staff gave an overview of rule 8.1b of the Pharmaceutical Schedule. Under this rule, District Health Board hospitals may give any pharmaceutical use within a paediatric oncology/haematology service for the treatment of cancer. This is a 'legacy' rule and Pharmac staff noted that we haven't articulated well why the rule should or should not exist. The planned two-stage consultation is to help understand the environment the rule exists within and determine if there is an issue. The first consultation may, or may not, result in a proposal to change to the rule. There would be a second consultation on any proposals for change.

Out of scope

Out of scope

Out of scope

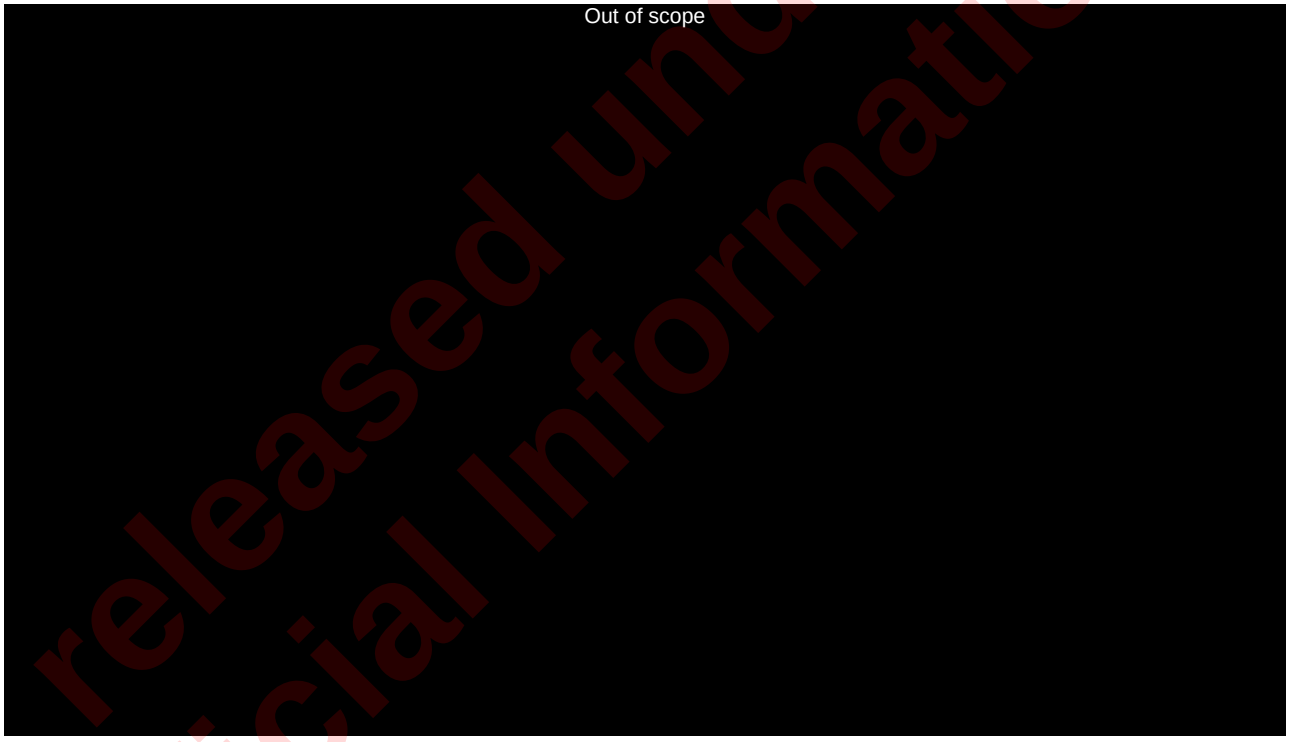
Members noted that although the rule was not equal, it did provide equitable outcomes – therefore the rule itself is equitable. Members also noted the importance of prioritising children.

Members noted things to consider included whether there was the ability for pharmaceutical companies to 'abuse' the rule and the line between paediatrics and adolescents. Members also noted that Pharmac should be mindful of timing should there be any changes to the rule.

Members noted that most Māori are aged under 25 and there needs to be higher considerations of these implications. Members suggest that biology be a marker point rather than a calendar age.

Members noted that Pharmac should focus on paediatric oncologists' opinions and knowledge here given this was their area of expertise.

Members in general were supportive of the rule as it was currently.



10. Karakia and meeting close

The meeting was closed with a karakia.