From: Rachel Read
Sent: Monday, 3 May 2021 12:11 pm
To: Laura Seary <Withheld under section 9(2)(a)>
Cc: Peter Jane <Withheld under section 9(2)(a)>
Subject: 2021-05-03 Paediatric oncology medicines key points for Ministers office

Hi Laura

Please find attached background information as requested

If you need anything further please let me know

Cheers Rachel

Rachel Read | Manager, Policy and Government Services | Engagement & Implementation

PHARMAC | Te Pātaka Whaioranga | PO Box 10 254 | Level 9, 40 Mercer Street, Wellington
DDI: Withheld under | P: +64 4 460 4990 | M: Withheld under section
www.pharmac.govt.nz

Paediatric oncology treatment messages

Umbrella message

How PHARMAC funds paediatric oncology treatments is currently inconsistent with how all other treatments are funded through the Combined Pharmaceutical Budget.

PHARMAC is in the early stages of reviewing how it funds paediatric cancer medicines to ensure that it is being transparent and fair.

Core messages

- PHARMAC is in the early stages of this review.
- Before any decisions are made, PHARMAC wants to talk with clinicians and paediatric oncologists. It will consult with clinical experts on its cancer treatments subcommittee (CATSoP) as well as paediatric oncology doctors in May.
- PHARMAC wants to ensure that all medicines currently being used in paediatric oncology can continue to be available. No medicines currently funded will be removed.
- The majority of paediatric oncology treatments are already on the Pharmaceutical Schedule. However, some treatments used for a very small number of children (approximately 50 to 100 children per year) are not listed on the Schedule. This is a historical inconsistency which has existed since cancer medicines were managed by the District Health Board hospitals. PHARMAC now wants to bring funding of those paediatric oncology treatments into alignment with the Pharmaceutical Schedule.
- While it is ultimately PHARMAC's role to decide which medicines are publicly funded for New Zealanders, it works with a number of external experts when making these tough decisions. PHARMAC is guided by robust evidence and the expertise of clinicians, the healthcare sector and feedback from consumers.

Background

Why is a different approach taken for paediatric oncology treatments?

Before PHARMAC's involvement, DHBs each undertook their own assessments and decision-making on paediatric cancer treatments, resulting in some inconsistency in the range of cancer treatments that could be accessed in different parts of the country. A key rationale for PHARMAC's involvement was to support a more consistent approach to pharmaceutical cancer treatment across the country.

In 2001, when PHARMAC was preparing to take on the role of managing pharmaceutical cancer treatments, discussions with oncologists and DHBs highlighted some complexities in relation to paediatric oncology treatments. These complexities made normal Pharmaceutical Schedule listings problematic in some cases. They included the specialized nature of some of these treatments, often used differently in children than in adults; the small number of patients each year for most indications; and that some of the medicines and indications were unregistered. At the time, PHARMAC was not routinely listing unapproved medicines in the Schedule. Paediatric oncology also had its own funding stream, creating potential issues for financial management of oncology services.

PHARMAC was not able to fully resolve these issues at the time, and in 2005, it was agreed that an Exceptional Circumstances funding mechanism would be an appropriate solution. This resulted in the current funding pathway for paediatric cancer medicines (Rule 8.1b of the Pharmaceutical Schedule).

Current work

PHARMAC first commenced work on reviewing the funding pathway for paediatric cancer medicines in 2019, and undertook analysis of the numbers of patients, types of treatments, and the cost of treatments being accessed through this pathway.

The data showed that the majority of medicines dispensed through the paediatric cancer pathway are already on the Pharmaceutical Schedule and available to paediatric oncology patients. Because of this, the work was not progressed with urgency.

Following a complaint to the Human Rights Commission, on 12 February 2021, PHARMAC confirmed with Office of Human Rights Proceedings that it was reviewing the funding arrangements for paediatric cancer treatments, to bring funding in line with PHARMAC's usual processes.

PHARMAC is currently reviewing this funding pathway and assessing it against alternative options for funding paediatric oncology treatments. This will be informed by an analysis of the latest available data on the treatments currently used. The recommended option will need to satisfy several principles, which include ensuring that all medicines currently used continue to be available, continuity of care for current patients, and prescribers being able to continue operating as they do now.

PHARMAC intends to consult on any proposed changes to current funding arrangements.

From: Rachel Read
Sent: Monday, 3 May 2021 4:37 pm
To: Laura Seary <Withheld under section 9(2)(a)>
Cc: Peter Jane <Withheld under section 9(2)(a)>
Subject: further media statements on paediatric oncology

Hi Laura

To keep you in the loop, there has been further media interest today in paediatric cancer medicines.

PHARMAC sent the following statement, attributed to Sarah Fitt, to both TV1 and 3 for tonight's news.

PHARMAC is in the very early stages of reviewing how we fund paediatric cancer treatments. This is because how we fund these treatments is inconsistent with how we fund all other medicines through the Combined Pharmaceutical Budget. We want to ensure that we are being transparent and fair to all, and that does mean reviewing how we fund these medicines. No decisions have been made.

I want to reassure New Zealanders that medicines being used for paediatric cancer treatments now will continue to be available for our children and young people. Medicines funded now will continue to be funded.

Our next step is to consult with our Cancer Treatments Subcommittee as well as seek feedback from oncologists and haematologists working with children and young people. Following those discussions, we will consult more widely with the rest of the healthcare sector and the public.

We take our decision-making responsibility seriously. The wellbeing of New Zealanders is at the heart of our work, and we want to provide Kiwis with the medicines they need to live healthy lives.

Following that Sarah did an interview with Jenna Lynch (Newshub) where she clarified the following points (largely because a patient advocate went on record saying this would mean child cancer patients would stop receiving treatment):

- *First off, I want to address the idea that we will be stopping funding any child cancer treatments currently in use. I want to reassure New Zealanders that medicines being used for paediatric cancer treatments now will continue to be available for our children and young people. Any suggestion to the contrary is just fearmongering.*
- *We are in the very early stages of reviewing how we fund these treatments. We are doing this to ensure that we continue to be fair and giving kiwis the best medicines to help them with their lives. What won't change is that any treatments currently in use will stay in use.*
- *When we make decisions on medicines to fund, we talk with our expert external committees, those in the healthcare sector and are guided by the science. We also listen to the public.*
- *Our aim is to provide Kiwis with the medicines they need to live healthy lives. We will continue to do that.*

Regards Rachel

Rachel Read | Manager, Policy and Government Services | Engagement & Implementation

PHARMAC | Te Pātaka Whaioranga | PO Box 10 254 | Level 9, 40 Mercer Street, Wellington
DDI: Withheld under | P: +64 4 460 4990 | M: Withheld under section
www.pharmac.govt.nz

PHARMAC
TE PĀTAKA WHAIORANGA

BRIEFING

Funding of Paediatric Cancer Treatments

Date: **5 May 2021**

To: **Hon Andrew Little (Minister of Health)**

Copies to

Minister of Health
Director General of Health
PHARMAC Board
Lead DHB Chief Executive, Pharmaceuticals
Principal Advisor, Governance and Crown Entities, Ministry of Health
PHARMAC Review Committee

Recommendations

It is recommended you:

- **note** the contents of this briefing

Contact(s)

Sarah Fitt, Chief Executive
Alison Hill, Director Engagement and Implementation

Withheld under
Withheld under

Purpose

PHARMAC is in the early stages of reviewing how it funds paediatric cancer treatments to ensure that it is being transparent and fair. The project has recently received significant media attention. This briefing provides you with background information on the project, and an update on current progress.

Background

Paediatric cancer treatments are treated differently by PHARMAC

PHARMAC takes a different approach to paediatric cancer treatments (PCTs) than to how it funds treatments for other conditions.

This difference is due to rule 8.1b of the Pharmaceutical Schedule. Under this rule, District Health Board hospitals (DHBs) may give (and will be eligible to receive a subsidy for) any medicine for use within a paediatric oncology/haematology service for the treatment of cancer. This is known as the 'PCT pathway.'

PHARMAC does not require medicines used under the PCT pathway to undergo the same decision-making process that is required for normal listings, or for other applications under the Exceptional Circumstances Framework.

That means, under the PCT pathway, paediatric oncologists and/or paediatric haematologists in New Zealand can give (within a DHB paediatric oncology/haematology service), and receive subsidy for, any pharmaceutical to children with cancer for the treatment of their cancer, regardless of the evidence base or cost-effectiveness.

There is no separate budget allocation for paediatric cancer treatments. All pharmaceutical treatments for paediatric cancer patients (whether for treatments listed on the Pharmaceutical Schedule or for treatments approved under the Exceptional Circumstances framework) are funded from the Combined Pharmaceutical Budget (CPB). The number of patients accessing paediatric cancer treatments is relatively small. Approximately 400 patients per year access paediatric cancer treatments, which includes those listed on the Schedule. The annual cost per annum is approximately $3 million.

As with pharmaceuticals accessed through the Exceptional Circumstances framework, PHARMAC manages an administrative process to provide access to a subsidy from the CPB. The process to access funding from the PCT pathway is initiated by a hospital pharmacist or a health professional working in a relevant paediatric oncology or haematology service, through completion of a notification form that is submitted to PHARMAC.

The difference is due to historical reasons

Before PHARMAC's involvement, DHBs each undertook their own assessments and decision-making on paediatric cancer treatments, resulting in some inconsistency in the range of cancer medicines that could be accessed in different parts of the country. A key rationale for PHARMAC's involvement was to support a more consistent approach to cancer treatment across the country.

In 2001, when PHARMAC was preparing to take on the role of managing pharmaceutical cancer treatments, discussions with oncologists, haematologists and DHBs highlighted some complexities in relation to paediatric cancer treatments. These complexities made normal Schedule listings problematic in some cases. They included the specialised nature of some of these treatments, often used differently in children than in adults; the small number of patients each year for most indications; some patients being enrolled in international clinical trials and that some of the medicines and indications were unregistered.

Additionally, paediatric cancer had its own funding stream, creating potential issues for financial management of oncology/haematology services. This is now no longer the case.

PHARMAC was not able to fully resolve these issues at the time, and in 2005, it was agreed that an Exceptional Circumstances funding mechanism would be an appropriate solution. This resulted in Rule 8.1b of the Pharmaceutical Schedule.

The difference has created an inconsistency with medicines for other conditions

The different approach taken to funding of paediatric cancer treatments has resulted in an inconsistency with the funding mechanisms for other treatments through the Schedule, including other paediatric treatments and adult cancer services.

Additionally, although expenditure on paediatric cancer treatments is relatively low, it has been increasing in recent years. Continuation of the current funding approach may cause a risk to the CPB if cost increases cannot be predicted or contained, particularly considering the recent development of expensive treatments for childhood cancers, such as CAR T-cell therapy.

That inconsistency was an aspect of a complaint to the Human Rights Commission

In May 2020, Ms Fiona Tolich lodged a complaint with the Human Rights Commission (HRC) that focused on funding for spinal muscular atrophy treatments, including nusinersen (Spinraza). The complaint highlighted the inconsistency between PHARMAC's funding for paediatric cancer treatments and treatments for other paediatric conditions.

Work underway

Work has been underway since 2019

Aware of the inconsistency, PHARMAC first commenced work on reviewing the PCT pathway in 2019. Analysis was undertaken of the numbers of patients, types of treatments, and the cost of treatments being accessed through this pathway.

The data showed that most medicines dispensed through the PCT pathway are already on the Pharmaceutical Schedule and available to paediatric cancer patients. Because of this, and due to the impact of COVID-19, the work was not progressed with urgency.

Work has commenced again since the HRC complaint

PHARMAC has again commenced the review in light of the HRC complaint. We are currently assessing the existing PCT pathway against alternative options. This is being informed by an analysis of the latest available data on the treatments currently used.

Any change to the PCT pathway will need to satisfy several principles, which include:

- ensuring that all medicines currently used continue to be available,
- ensuring continuity of care for current patients,
- being consistent with how medicines for other conditions are treated,
- prescribers being able to continue operating as they do now, and
- being sustainable and responsive.

The project has recently received significant media attention

On 12 February 2021, PHARMAC confirmed with Office of Human Rights Proceedings that it was reviewing the funding arrangements for paediatric cancer treatments, with a view to bringing funding into line with PHARMAC's usual processes.

Following an interview between Ms Tolich and Radio New Zealand journalist, Guyon Espiner, PHARMAC's review of the PCT pathway has generated significant media attention.

In response, PHARMAC has emphasised that no final decisions on the future of the PCT pathway have been made, and regardless of these decisions, all treatments currently used for paediatric cancer will continue to be available.

Next steps

As part of reviewing how PHARMAC funds paediatric cancer treatments, we expect to engage with stakeholders on potential options, including PTAC cancer subcommittee members, paediatric oncologists and haematologists, patients and their families, and advocacy groups from late May 2021. We would consult publicly before any decisions are made.

Excerpt of PHARMAC weekly key messages

Updated Thursday 6 May 2021

Paediatric oncology medicines

Background

The approach PHARMAC takes to funding paediatric oncology treatments is inconsistent with funding mechanisms for other treatments through the Combined Pharmaceutical Budget. This is due to rule 8.1b of the Pharmaceutical Schedule (PCT pathway). We are reviewing this funding pathway and assessing it against alternative options for funding paediatric oncology treatments. There has been widespread interest from media and on social media.

Communications approach

Sarah Fitt has taken interviews with RNZ and Newshub. Reactive messages have been prepared for any further questions. Prior to any consultation a proactive communications plan will be drafted.

Umbrella message

- Medicines being used for paediatric cancer treatments now, will continue to be available for our children and young people. Any suggestion to the contrary is just fearmongering. Any treatments currently in use will stay in use.

Core messages

- How we fund paediatric oncology treatments is currently inconsistent with how we fund all other treatments through the Combined Pharmaceutical Budget. We want to ensure that we are being transparent and fair and that means reviewing how we fund these medicines.
- We are in the very early stages of reviewing how we fund these treatments. We are doing this to ensure that we continue to be fair and give Kiwis the best medicines to help them with their lives.
- When we make decisions on medicines to fund, we talk with our expert external committees, those in the healthcare sector and are guided by the science. We also listen to the public.
- The majority of paediatric oncology treatments are already on the Pharmaceutical Schedule. However, some treatments used for a very small number of patients, are not listed on the Schedule. This is a historical inconsistency which has existed since cancer medicines were managed by the District Health Board hospitals. We need to work out how to fairly and consistently deal with paediatric oncology treatments compared with other medicines for other people.

PHARMAC
TE PĀTAKA WHAIORANGA

CONFIDENTIAL – PLEASE DO NOT DISTRIBUTE

BRIEFING TO THE CANCER TREATMENTS SUBCOMMITTEE OF PTAC

To: Cancer Treatments Subcommittee of PTAC (CaTSoP)

From: PHARMAC

Update on the paediatric cancer treatments review

Questions to the Subcommittee Members for discussion

1. What are your views regarding the current inequities between paediatric patients with cancer and other patient groups?

2. Are you aware of how decisions regarding the treatment of paediatric patients with cancer are currently made?

3. What proportion of paediatric patients with cancer are treated as part of a clinical trial?

4. Withheld under section 9(2)(f)(iv)

   Withheld under section 9(2)(f)(iv)

   Withheld under section 9(2)(f)(iv)

   Withheld under section 9(2)(f)(iv)

   Withheld under section 9(2)(f)(iv)

   Withheld under section 9(2)

   Withheld under section

   c. What are some of the key challenges that PHARMAC could face with any change to the current arrangement?

      i. Reputationally?

      ii. From a process perspective? (e.g., Level of evidence, Medsafe approval etc.)

   d. What are the key considerations that PHARMAC should consider during implementation of such changes? (e.g. timing of changes, engagement regarding said changes)

5. Withheld under section 9(2)(f)(iv)

   Withheld under section 9(2)(f)(iv)

6. Who else should we be asking, at this early stage, in order to develop a solution that works best for all?

   a. Who can we best engage with for Māori clinical and other advice in this area?

b. What and who might be the most appropriate patient support and advocacy groups to engage with?

Purpose of Paper

PHARMAC is in the early stages of reviewing how it funds paediatric pharmaceutical cancer treatments (PCTs) to ensure we are being transparent and fair..

This briefing provides you with background information on the review and our current progress and seeks your feedback on our early thinking.

Background

Paediatric PCTs are treated differently by PHARMAC

PHARMAC takes a different approach to paediatric PCTs than to how it funds treatments for other conditions.

This difference is due to rule 8.1b of the Pharmaceutical Schedule. Under this rule, District Health Board hospitals (DHBs) may give (and will be eligible to receive a subsidy for) any medicine for use within a paediatric oncology/haematology service for the treatment of cancer. This is known as the 'paediatric PCT pathway.'

PHARMAC does not require medicines used under the paediatric PCT pathway to undergo the same decision making process that is required for Pharmaceutical Schedule listings or for other applications under PHARMAC's Exceptional Circumstances Framework, such as Named Patient Pharmaceutical Assessment (NPPA) applications.

That means that, under the paediatric PCT pathway, paediatric oncologists and/or paediatric haematologists in New Zealand can give (within a DHB paediatric oncology/haematology service) any pharmaceutical to children for the treatment of their cancer and it will be funded. This is regardless of the extent of very high need, evidence of benefit and whether or not it is cost-effective.

There is no separate budget allocation for paediatric PCTs. All pharmaceutical treatments for paediatric patients with cancer, including those listed on the Pharmaceutical Schedule and those funded via the paediatric PCT mechanism, are funded from the Combined Pharmaceutical Budget (CPB).

As with pharmaceuticals funded through the Exceptional Circumstances Framework, PHARMAC manages an administrative process to provide funding from the Combined Pharmaceutical Budget (CPB) for paediatric PCTs. The process to access funding from the paediatric PCT pathway is initiated by a DHB hospital pharmacist or a health professional working in a relevant DHB paediatric oncology or haematology service, through completion of a notification form that is submitted to PHARMAC.

PHARMAC will then authorise the issuing of an approval number that will allow the dispensing pharmacy to claim for the cost of the paediatric PCT from the CPB. Approvals are limited to treatments of the actual cancer. Approvals are granted to allow claiming for the full cost of the cancer treatment (excluding supportive care treatments) and are generally granted for a five-year period, and may be extended on application (only 1% paediatric PCT approvals have required renewal).

The difference is historical

Before PHARMAC's involvement, DHBs each undertook their own assessments and decision-making on cancer treatments, resulting in some inconsistency in the range of

cancer medicines that could be accessed in different parts of the country. A key rationale for PHARMAC's involvement was to support a more consistent approach to the funding of cancer medicines across the country.

When PHARMAC was initially preparing to take on the role of PCTs, discussions with oncologists, haematologists and DHBs highlighted some complexities in relation to paediatric PCTs. These complexities made regular Pharmaceutical Schedule listings problematic in some cases.

PHARMAC was not able to fully resolve these issues at the time, and in 2005 it was agreed that a separate funding mechanism specific to paediatric cancer treatments would be an appropriate solution. This resulted what is now Rule 8.1b of the Pharmaceutical Schedule – the "paediatric PCT pathway". It was intended at the time of creating Rule 8.1b that paediatric cancer treatments would remain outside of the usual funding pathways until a time that it needed to be reconsidered.

The different approach taken to the funding of paediatric PCTs has resulted in an inconsistency with the funding mechanisms for other treatments through the Schedule, including for other paediatric treatments and for adult cancer services.

The inconsistency was an aspect of a complaint to the Human Rights Commission

In May 2020, a complaint was lodged with the Human Rights Commission (HRC) that focused on the funding of nusinersen (Spinraza) for spinal muscular atrophy. The complaint highlighted the inconsistency between PHARMAC's mechanism of funding for paediatric PCTs and treatments for other paediatric conditions. PHARMAC had already commenced work to address the inconsistency at the time of the complaint.

Complexity of paediatric PCTs

Some of the complexities regarding paediatric PCTs are listed below:

- They are used in specialised cases
- They are often used differently in children compared to adults
- There are a small number of patients each year receiving treatment for most indications
- Some patients have access to, and are enrolled in, international clinical trials
    - We note that these are not standard supplier-led clinical trials, but rather trials designed to identify the most appropriate dose of a treatment in this patient population. In these trials, in most cases, the standard of care is not funded
        - Accreditation and involvement in these trials is considered important for these clinicians, and there are many people involved in maintaining this accreditation
    - We note that there are additional benefits of access to treatment beyond the pharmaceutical being applied for, and funded, by PHARMAC (e.g. medicines provided free of charge)
- Timely access to treatments is required for these patients and there is a need to be able to move quickly to make these available

- Some of the medicines and indications are not Medsafe approved

- Working definitions of "paediatric" age groups (to the extent that medicines are provided under paediatric PCT pathways) differ across different cancer types and the different configurations of the two main centre paediatric cancer services.

We understand that access to paediatric PCTs within each of the two main centres (Auckland and Christchurch) is peer reviewed by multidisciplinary teams. At this time, we are not certain if the same treatments are being accessed for the same/similar patients in both centres.

Current usage of medicines in paediatric oncology

The data we have indicates that there were approximately 390 paediatric patients that accessed treatment for cancer in the 2019/2020 financial year. We note that this is not incident patients and may therefore be higher than the patient numbers that have been communicated to us previously (150 patients per year, with two thirds being from Auckland). We acknowledge that there may be some miscoding of treatments that contribute to the number of patients in this data set.

The annual cost for all treatments in the 2019/2020 financial year was approximately $3 million, however this is increasing with the advent of novel, more expensive treatments.

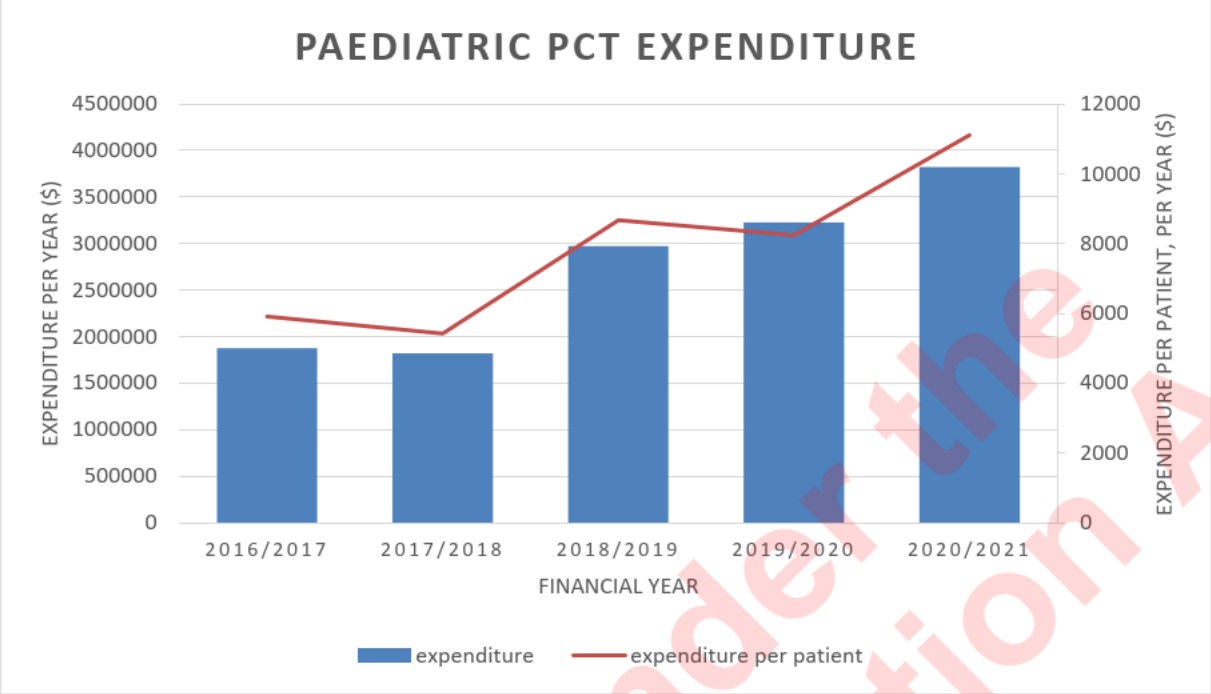
We understand that the vast majority of treatments used are currently listed on the Pharmaceutical Schedule, although it is not known whether all these patients would meet the current eligibility criteria for these medicines.

A list of the treatments used for paediatric cancers over the past five years (including those accessed through the paediatric PCT pathway and those accessed directly through the Pharmaceutical Schedule listings), and numbers of patients for each, is shown in **Appendix 1**. This data includes all treatments used by patients, born after the year 2000, which could reasonably be assumed to be for the treatment of cancer.

Although expenditure on paediatric PCTs is relatively low, it has been increasing in recent years (**Figure 1**). The current financial year is likely to be even greater than that depicted, as this data is not complete **Figure 1**and is driven by newer products (e.g., blinatumomab).

As well as perpetuating an inconsistency between funding of treatments for paediatric and adult patients with cancer, and between paediatric patients with or without cancer, continuing the current funding approach to paediatric PCTs poses a financial risk to the CPB as more expensive medicines become available and used without PHARMAC oversight.

Figure 1: Expenditure on paediatric PCTs from 2016/17 – 2020/21



Work underway

Work has been underway since 2019

Aware of the inconsistency and the risk to the CPB with the advent of new technologies such as Chimeric Antigen Receptor (CAR) T-cell therapy, PHARMAC began a review of the paediatric PCT pathway in early 2019. Analysis was undertaken of the numbers of patients, types of treatments and the cost of treatments being accessed through this pathway.

As noted above, the data showed that most medicines dispensed through the paediatric PCT pathway are already listed on the Pharmaceutical Schedule. Many of these are open listed currently and are likely available to paediatric patients with cancer. We note however, that there are some paediatric cancer treatments, currently listed on the schedule that are not available to paediatric patients with cancer.

Although the analysis did identify a trend towards expenditure increases, the work again highlighted the complexities of paediatric PCTs noted above. This, combined with the impact of COVID-19, resulted in the work being put on hold.

Work has commenced again since the HRC complaint

PHARMAC has recommenced the review of the paediatric PCT pathway in light of the HRC complaint. We are currently assessing the existing pathway against alternative options. This is being informed by an analysis of the latest available data on the treatments currently used through the paediatric PCT pathway.

PHARMAC has developed a set of principles that any change to the paediatric PCT pathway will need to satisfy. These include:

- ensuring that all medicines currently used by this patient group continue to be available
- ensuring continuity of care for current patients

- being consistent with how medicines for other conditions are treated
- prescribers being able to continue operating as similarly as possible to how they do now
- being sustainable and responsive.

We have started to develop options

Using the principles outlined above, and our understanding of the medicines currently used via the paediatric PCT pathway, PHARMAC has started to look at alternative options.

Our analysis to date has shown that medicines funded via the paediatric PCT pathway can be divided into three groups, each needing a different solution. These groups are:

- medicines listed on the Pharmaceutical Schedule
- medicines listed on the Pharmaceutical Schedule but not available to paediatric oncology patients
- medicines not listed on the Pharmaceutical Schedule.

We also need to consider options for how we could assess future Pharmaceutical Schedule funding applications for medicines used in paediatric cancers, as well as applications for medicines for individual paediatric patients with cancer (including in the context of a clinical trial).

There are other complexities that will need to be factored into our options development, including Withheld under section 9(2)(f)(iv)

Withheld under section 9(2)(f)(iv)

Withheld under section 9(2)(f)(iv)

Withheld under section 9(2)(f)(iv)

Withheld under section 9(2)(f)(iv)

Withheld under section 9(2)(f)(iv)

Withheld under section 9(2)(f)(iv)

Withheld under section 9(2)(f)(iv)

Withheld under section 9(2)(f)(iv)

Withheld under section 9(2)(f)(iv)

We are also aware that there are medicines currently provided to paediatric patients with cancer that do not show up in the paediatric PCT data, for example, medicines given as part of a clinical trial or free-stock programme. Withheld under section 9(2)(f)(iv)

Withheld under section 9(2)(f)(iv)

Withheld under section 9(2)(f)(iv)

Withheld under section 9(2)(f)(iv)

Withheld under section 9(2)(f)(iv)

Withheld under section 9(2)(f)(iv)

Figure 2: Potential workstreams

We would like your feedback

We are still at the early stages of this review. We want to take the necessary time needed to find the right solution, rather than rushing to implement what may eventuate to be an undesirable outcome.

Before we progress any further, we would like to hear your thoughts on the potential alternative. We acknowledge that there could also be other options available to PHARMAC that have not been actively explored yet.

Once we have received your feedback, we will expand our engagement as our thinking develops. As well as your feedback on the potential alternative solution, we would like to hear your views on who else we should be engaging with.

Next steps

We plan to use your feedback to:

- make refinements to an alternative option to the current status quo
- inform who, when and how we should engage with the wider community, including:
    - paediatric oncologists and haematologists in Auckland and Canterbury
    - Leukaemia & Blood Cancer New Zealand and other patient advocacy groups

- Medsafe
- representatives for Māori and Pacific patients, communities, health professionals and health providers.

Following this, we would be seeking to undertake a full public consultation. We will be sure to keep you updated on this project as it progresses.

Appendices

Appendix 1: Data on usage and cost of paediatric PCT's in the previous five financial years

Excerpt of PHARMAC weekly key messages

Updated Thursday 20 May 2021

Paediatric oncology medicines

Background

The funding pathway for paediatric oncology treatments is inconsistent with funding mechanisms for other treatments through the Combined Pharmaceutical Budget. This is an approach that was inherited from DHBs when PHARMAC was transferred responsibility for decision-making about cancer treatments in 2010.

We're reviewing this funding pathway and assessing it against alternative options for funding paediatric oncology treatments. There has been widespread interest from media and on social media.

Communications approach

Sarah Fitt has taken interviews with RNZ and Newshub. Reactive messages have been prepared for any further questions. Prior to any consultation a proactive communications plan will be drafted.

Main message

- Medicines being used for paediatric cancer treatments now, will continue to be available for our tamariki and rangatahi. Any suggestion to the contrary is just fearmongering.

Core messages

- How we fund paediatric oncology treatments is inconsistent with how we fund all other treatments through the Combined Pharmaceutical Budget. We want to ensure that we're being fair, so we are reviewing how we fund these medicines.
- We've not made any decisions about what would change, or if there would be changes. We are in the early stages of talking to clinical experts across the country about this. As we work through potential options, we would consult widely and seek feedback from the public.
- The majority of paediatric oncology treatments are already on the Pharmaceutical Schedule – and will stay there. This means they are already approved funded medicines. However, some treatments used for a very small number of patients, aren't listed on the Schedule. This is a historical inconsistency which has existed since cancer medicines were managed by the District Health Board hospitals.
- We need to work out how to fairly and consistently deal with paediatric oncology treatments compared with other medicines for other conditions.

Excerpt of PHARMAC weekly key messages

Updated Thursday 27 May 2021

Paediatric oncology medicines

Background

The funding pathway for paediatric oncology treatments is inconsistent with funding mechanisms for other treatments through the Combined Pharmaceutical Budget. This is an approach that was inherited from DHBs when PHARMAC was transferred responsibility for decision-making about cancer treatments in 2010.

We're reviewing this funding pathway and assessing it against alternative options for funding paediatric oncology treatments. There has been widespread interest from media and on social media.

Communications approach

Sarah Fitt has taken interviews with RNZ and Newshub. Reactive messages have been prepared for any further questions. A proactive communications plan is being drafted.

Main message

- Medicines being used for paediatric cancer treatments now, will continue to be available for our tamariki and rangatahi. Any suggestion to the contrary is just fearmongering.

Core messages

- How we fund paediatric oncology treatments is inconsistent with how we fund all other treatments through the Combined Pharmaceutical Budget. We want to ensure that we're being fair, so we are reviewing how we fund these medicines.
- We've not made any decisions about what would change, or if there would be changes. We are in the early stages of talking to clinical experts across the country about this. As we work through potential options, we would consult widely and seek feedback from the public.
- The majority of paediatric oncology treatments are already on the Pharmaceutical Schedule – and will stay there. This means they are already approved funded medicines. However, some treatments used for a very small number of patients, aren't listed on the Schedule. This is a historical inconsistency which has existed since cancer medicines were managed by the District Health Board hospitals.
- We need to work out how to fairly and consistently deal with paediatric oncology treatments compared with other medicines for other conditions.
- This week we talked with our Cancer Treatments subcommittee to discuss the issue and to hear their perspectives. We will be talking with paediatric oncology specialists in the next month.
- Hearing how medicines impact the lives of New Zealanders is important in helping us understand what medicines Kiwis think we should be funding.

Excerpt of PHARMAC weekly key messages

Updated Thursday 3 June 2021

Paediatric oncology medicines

Background

The funding pathway for paediatric oncology treatments is inconsistent with funding mechanisms for other treatments through the Combined Pharmaceutical Budget. This is an approach that was inherited from DHBs when PHARMAC was transferred responsibility for decision-making about cancer treatments in 2010.

We're reviewing this funding pathway and assessing it against alternative options for funding paediatric oncology treatments. There has been widespread interest from media and on social media.

Communications approach

Sarah Fitt has taken interviews with RNZ and Newshub. Reactive messages have been prepared for any further questions. A proactive communications plan is being drafted.

Main message

- Funded medicines being used for paediatric cancer treatments now, will continue to be available for our tamariki and rangatahi. Any suggestion to the contrary is just fearmongering.

Core messages

- How we fund paediatric oncology treatments is inconsistent with how we fund all other treatments through the Combined Pharmaceutical Budget. We want to ensure that we're being fair, so we are reviewing how we fund these medicines.
- We've not made any decisions about what would change, or if there would be changes. We are in the early stages of talking to clinical experts across the country about this. As we work through potential options, we would consult widely and seek feedback from the public.
- The majority of paediatric oncology treatments are already on the Pharmaceutical Schedule – and will stay there. This means they are already approved funded medicines. However, some treatments used for a very small number of patients, aren't listed on the Schedule. This is a historical inconsistency which has existed since cancer medicines were managed by the District Health Board hospitals.
- We need to work out how to fairly and consistently deal with paediatric oncology treatments compared with other medicines for other conditions.
- This week we talked with our Cancer Treatments subcommittee to discuss the issue and to hear their perspectives. We will be talking with paediatric oncology specialists in the next month.
- Hearing how medicines impact the lives of New Zealanders is important in helping us understand what medicines Kiwis think we should be funding.

PHARMAC
TE PĀTAKA WHAIORANGA

Paediatric Cancer Treatments

Update to Te Aho o Te Kahu

Logan Heyes
Senior Therapeutic Group Manager

22 June 2021

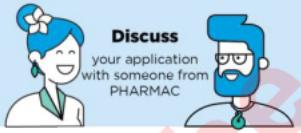
The journey of a funding application

A medicine supplier, health professional or an everyday New Zealander can apply for a medicine or medical device to be funded.

PHARMAC Factors for Consideration are used throughout

Prepare your application by collating all relevant information

Discuss your application with someone from PHARMAC



Submit your application via PHARMConnect

Application Tracker See your application status online

Review PHARMAC reviews and evaluates the evidence, including others' submissions on the same medicine or medical device

Advice PHARMAC receives recommendations from independent clinical experts from across New Zealand's health care sector who sit on the Pharmacology and Therapeutics Advisory Committee (PTAC) and its subcommittees

PTAC

Subcommittees

Recommendations

Assess PHARMAC considers the clinical advice and assesses the medicine. Research and economic analysis take place

HIGH

Ranking

LOW

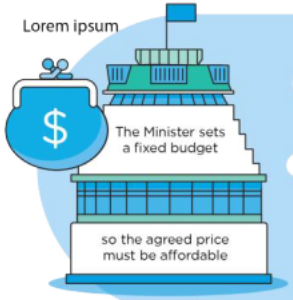
Compare options PHARMAC compares and ranks medicines

Prioritise A prioritised list of medicines for funding is created

Identify PHARMAC identifies which medicines on the list to take forward

Negotiate PHARMAC negotiates price with suppliers

Lorem ipsum



Provisional agreement PHARMAC and the supplier confirm a provisional agreement

Consult We ask New Zealanders what they think

Consider submissions

Final changes PHARMAC staff make any changes necessary following the consultation submissions

Final decision The PHARMAC board or delegate makes a final decision

Notification PHARMAC notifies the decision to health professionals and the public

Listed If approved, the medicine or medical device is listed on the Pharmaceutical Schedule

PHARMAC
TE PĀTAKA WHAIORANGA

For further information visit **pharmac.govt.nz**

The process set out in this diagram is intended to be indicative of the process that may follow where a supplier or other applicant wishes a pharmaceutical to be funded on the Pharmaceutical Schedule. PHARMAC may, at its discretion, adopt a different process or variations of the process. For example, we decide whether or not it is appropriate to undertake consultation on a case-by-case basis.

PHARMAC Factors for Consideration are used throughout

Application Tracker See your application status online

Paediatric cancer treatments

- Pharmac currently takes a different approach to paediatric cancer treatments compared to how it funds treatments for other conditions.
- This difference is due to rule 8.1b of the Pharmaceutical Schedule.
  - This enables the funded use of any medicine for use within a paediatric oncology or haematology service for the treatment of cancer.
    - The service has its own way of prioritising access to these medicines – peripherally to Pharmac
- **This is known informally as the 'paediatric PCT pathway'.**

The difference is historical

- DHBs used to undertake their own assessments and decision-making on cancer treatments.
  - This created some inconsistency across the country.
- Pharmac became involved to support a more consistent approach to funding cancer medicines across the country.
  - A separate funding mechanism was implemented in 2005 due to the complexities of paediatric cancer treatments.

PHARMAC
TE PĀTAKA WHAIORANGA

The provision of paediatric cancer treatments is complex

- Clinical trials
- Access needs to be timely
- This patient group has been served well by virtue of this pathway
- It is important to consider these patients by indication rather than age
- Many of the medicines are not Medsafe approved
- Making paedatric cancer treatments fit the wider Pharmac model is difficult

Work has been underway for a while

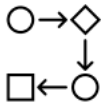
- Work to understand the complexity started in 2019 and has been recommenced recently
  - Inconsistent with access to medicines for other patient groups
  - Inability to understand future costs of medicines for this patient group

Understanding the current situation

- We have spoken with CaTSoP to understand the complexity further and what may be needed if an alternative approach were to be implemented
- We will be engaging directly with the sector in the coming weeks to understand the status quo and the impact of any change further

Guiding principles for alternative options

ensuring that all medicines currently used by this patient group continue to be available for current and new patients



ensuring compatibility with Pharmac's assessment and decision-making processes for other medicines

ensuring that prescribers can continue to operate as similarly as possible to how they do now

How can we work together to understand and understand and address this?

Understanding the status quo

Aligning with treatments for other patient groups

Engagement and identification of who to engage with prior to consideration of any change

PHARMAC
TE PĀTAKA WHAIORANGA

Excerpt of MEMORANDUM TO MEDICAL ONCOLOGY WORK GROUP

From: PHARMAC

Date: June 2021

5. Paediatric patient cancer treatment (PCT) work

Paediatric PCTs are treated differently by PHARMAC

PHARMAC takes a different approach to paediatric PCTs than to how it funds treatments for other conditions. This difference is due to rule 8.1b of the Pharmaceutical Schedule. Under this rule, District Health Board hospitals (DHBs) may give (and will be eligible to receive a subsidy for) any medicine for use within a paediatric oncology or haematology service for the treatment of cancer. This is known as the 'paediatric PCT pathway'.

The difference is historical

Before PHARMAC's involvement, DHBs each undertook their own assessments and decision-making on cancer treatments, resulting in some inconsistency in the range of cancer medicines that could be accessed by people in different parts of the country. A key reason for PHARMAC's involvement was to support a more consistent approach to funding cancer medicines across the country.

At that time, oncologists, haematologists, and DHBs highlighted some complexities in relation to paediatric PCTs. These complexities made regular Pharmaceutical Schedule listings problematic in some cases. PHARMAC was not able to fully resolve these issues at the time, and in 2005 it was agreed that a separate funding mechanism specific to paediatric cancer treatments would be an appropriate solution.

Work has been underway since 2019

PHARMAC began a review of the paediatric PCT pathway in early 2019. Although the analysis did identify a trend towards expenditure increases, the work again highlighted the complexities of the provision of paediatric PCTs.

PHARMAC has developed a set of principles to guide the development of any potential alternative options to the existing pathway for access to paediatric PCTs. These include:

- ensuring that all medicines currently used by this patient group continue to be available for current and new patients
- ensuring compatibility with PHARMAC's assessment and decision-making processes for other medicines
- ensuring that prescribers can continue to operate as similarly as possible to how they do now.

Current engagement

Recently we spoke with CaTSoP about this to identify and understand in more detail the issues and complexities associated with the current provision of paediatric cancer treatments. We intend to use these insights to help develop this thinking further. We will engage widely with external people as soon as we are in a position to do so.

Questions for MOWG

1) What are your views regarding the current inequities between paediatric people with cancer and other patient groups (eg. adult oncology, other paediatric patients)?

2) Who would be best to engage with for clinical and consumer advice in this area as it relates to Māori and Pacific children with cancer?

Excerpt of Weekly media and issues update

For the week ending 4 June 2021

Upcoming issues and events

Subject	Timeframe	Details
Review into paediatric oncology treatments	Ongoing	The approach to fund paediatric oncology treatments is inconsistent with funding mechanisms for other treatments through the Combined Pharmaceutical Budget. We are therefore in the early stages of reviewing how we fund paediatric oncology treatments. We talked with our Cancer Treatments Subcommittee on Wednesday 26 May and intend to speak with other key stakeholders, including paediatric oncologists, over the next few months. Following that, we intend to consult publicly on the proposed changes to the funding pathway before making any decisions. We are drafting a communications plan to support this work. This will be shared with stakeholders prior to any further release.

Excerpt of Weekly media and issues update

For the week ending 11 June 2021

Upcoming issues and events

Subject	Timeframe	Details
Review into paediatric oncology treatments	Ongoing	The approach to funding paediatric oncology treatments is inconsistent with mechanisms for other treatments through the Combined Pharmaceutical Budget. We are therefore in the early stages of reviewing how we fund paediatric oncology treatments. We talked with our Cancer Treatments Subcommittee on Wednesday 26 May and intend to speak with other key stakeholders, including paediatric oncologists, over the next few months. Following that, we intend to consult publicly on the proposed changes to the funding pathway before making any decisions. We are drafting a communications plan to support this work. This will be shared with stakeholders prior to any further release.

Excerpt of PHARMAC weekly key messages

Updated Thursday 10 June 2021

Paediatric oncology medicines

Background

The funding pathway for paediatric oncology treatments is inconsistent with funding mechanisms for other treatments through the Combined Pharmaceutical Budget. This is an approach that was inherited from DHBs when PHARMAC was transferred responsibility for decision-making about cancer treatments in 2010.

We're reviewing this funding pathway and assessing it against alternative options for funding paediatric oncology treatments. There has been widespread interest from media and on social media.

Communications approach

Sarah Fitt has taken interviews with RNZ and Newshub. Reactive messages have been prepared for any further questions. A proactive communications plan is being drafted.

Main message

- Funded medicines being used for paediatric cancer treatments now, will continue to be available for our tamariki and rangatahi. Any suggestion to the contrary is just fearmongering.

Core messages

- How we fund paediatric oncology treatments is inconsistent with how we fund all other treatments through the Combined Pharmaceutical Budget. We want to ensure that we're being fair, so we are reviewing how we fund these medicines.
- We've not made any decisions about what would change, or if there would be changes. We are in the early stages of talking to clinical experts across the country about this. As we work through potential options, we would consult widely and seek feedback from the public.
- The majority of paediatric oncology treatments are already on the Pharmaceutical Schedule – and will stay there. This means they are already approved funded medicines. However, some treatments used for a very small number of patients, aren't listed on the Schedule. This is a historical inconsistency which has existed since cancer medicines were managed by the District Health Board hospitals.
- We need to work out how to fairly and consistently deal with paediatric oncology treatments compared with other medicines for other conditions.
- We are talking to our Cancer Treatments subcommittee to discuss the issue and to hear their perspectives. We will be talking with paediatric oncology specialists in the next month.
- Hearing how medicines impact the lives of New Zealanders is important in helping us understand what medicines Kiwis think we should be funding.

PHARMAC
TE PĀTAKA WHAIORANGA

Excerpt of Weekly media and issues update

For the week ending 18 June 2021

Upcoming issues and events

Subject	Timeframe	Details
Review into paediatric oncology treatments	Ongoing	The approach to funding paediatric oncology treatments is inconsistent with mechanisms for other treatments through the Combined Pharmaceutical Budget. We are therefore in the early stages of reviewing how we fund paediatric oncology treatments. We have talked with our Cancer Treatments Subcommittee on Wednesday 26 May, and we are talking with the MoH Medical Oncology Working Group next week. We intend to speak with other key stakeholders, including paediatric oncologists, over the next few months. Following that, we intend to consult publicly on proposed changes to the funding pathway before making any decisions.

Excerpt of PHARMAC weekly key messages

Updated Thursday 17 June 2021

Paediatric oncology medicines

Background

The funding pathway for paediatric oncology treatments is inconsistent with funding mechanisms for other treatments through the Combined Pharmaceutical Budget. This is an approach that was inherited from DHBs when PHARMAC was transferred responsibility for decision-making about cancer treatments in 2010.

We're reviewing this funding pathway and assessing it against alternative options for funding paediatric oncology treatments. There has been widespread interest from media and on social media.

Communications approach

Sarah Fitt has taken interviews with RNZ and Newshub. Reactive messages have been prepared for any further questions. A proactive communications plan is being drafted.

Main message

- Funded medicines being used for paediatric cancer treatments now, will continue to be available for our tamariki and rangatahi. Any suggestion to the contrary is just fearmongering.

Core messages

- How we fund paediatric oncology treatments is inconsistent with how we fund all other treatments through the Combined Pharmaceutical Budget. We want to ensure that we're being fair, so we are reviewing how we fund these medicines.
- We've not made any decisions about what would change, or if there would be changes. We are in the early stages of talking to clinical experts across the country about this. As we work through potential options, we would consult widely and seek feedback from the public.
- The majority of paediatric oncology treatments are already on the Pharmaceutical Schedule – and will stay there. This means they are already approved funded medicines. However, some treatments used for a very small number of patients, aren't listed on the Schedule. This is a historical inconsistency which has existed since cancer medicines were managed by the District Health Board hospitals.
- We need to work out how to fairly and consistently deal with paediatric oncology treatments compared with other medicines for other conditions.
- We are talking to our Cancer Treatments subcommittee to discuss the issue and to hear their perspectives. We will be talking with paediatric oncology specialists in the next month.
- Hearing how medicines impact the lives of New Zealanders is important in helping us understand what medicines Kiwis think we should be funding.

Excerpt of PHARMAC weekly key messages

Updated Thursday 24 June 2021

Paediatric oncology medicines

Background

The funding pathway for paediatric oncology treatments is inconsistent with funding mechanisms for other treatments through the Combined Pharmaceutical Budget. This is an approach that was inherited from DHBs when PHARMAC was transferred responsibility for decision-making about cancer treatments in 2010.

We're reviewing this funding pathway and assessing it against alternative options for funding paediatric oncology treatments. There has been widespread interest from media and on social media.

Communications approach

Reactive messages have been prepared for any further questions. We will update key stakeholders once public consultation dates are agreed to.

Main message

- Funded medicines being used for paediatric cancer treatments now, will continue to be available for our tamariki and rangatahi. Any suggestion to the contrary is just fearmongering.

Core messages

- How we fund paediatric oncology treatments is inconsistent with how we fund all other treatments through the Combined Pharmaceutical Budget. We want to ensure that we're being fair, so we are reviewing how we fund these medicines.
- We've not made any decisions about what would change, or if there would be changes. We are in the early stages of talking to clinical experts across the country about this. As we work through potential options, we would consult widely and seek feedback from the public.
- The majority of paediatric oncology treatments are already on the Pharmaceutical Schedule – and will stay there. This means they are already approved funded medicines. However, some treatments used for a very small number of patients, aren't listed on the Schedule. This is a historical inconsistency which has existed since cancer medicines were managed by the District Health Board hospitals.
- We need to work out how to fairly and consistently deal with paediatric oncology treatments compared with other medicines for other conditions.
- We have talked to our Cancer Treatments subcommittee to discuss the issue and to hear their perspectives. We will be talking with paediatric oncology specialists in the next month.
- Hearing how medicines impact the lives of New Zealanders is important in helping us understand what medicines Kiwis think we should be funding.

PHARMAC
TE PĀTAKA WHAIORANGA

Excerpt of Weekly media and issues update

For the week ending 25 June 2021

Upcoming issues and events

Subject	Timeframe	Details
Review into paediatric oncology treatments	Ongoing	The approach to funding paediatric oncology treatments is inconsistent with mechanisms for other treatments through the Combined Pharmaceutical Budget. We are therefore in the early stages of reviewing how we fund paediatric oncology treatments. We talked with our Cancer Treatments Subcommittee on Wednesday 26 May, and this week we spoke with the MoH Medical Oncology Working Group . We intend to speak with other key stakeholders, including paediatric oncologists, over the next few months. Following that, we intend to consult publicly on any proposed changes to the funding pathway before making final decisions.

PHARMAC
TE PĀTAKA WHAIORANGA

Excerpt of Weekly media and issues update

For the week ending 16 July 2021

Upcoming issues and events

Subject	Timeframe	Details
Review into paediatric oncology treatments	Ongoing	The approach to funding paediatric oncology treatments is inconsistent with mechanisms for other treatments through the Combined Pharmaceutical Budget. We are therefore in the early stages of reviewing how we fund paediatric oncology treatments. We will consult publicly on any proposed changes to the funding pathway before making final decisions.

PHARMAC
TE PĀTAKA WHAIORANGA

Excerpt of Weekly media and issues update

For the week ending 23 July 2021

Upcoming issues and events

Subject	Timeframe	Details
Review into paediatric oncology treatments	Ongoing	The approach to funding paediatric oncology treatments is inconsistent with mechanisms for other treatments through the Combined Pharmaceutical Budget. We are reviewing how we fund paediatric oncology treatments.

PHARMAC
TE PĀTAKA WHAIORANGA

CONFIDENTIAL – PLEASE DO NOT DISTRIBUTE

BRIEFING TO THE PHARMACOLOGY AND THERAPEUTICS ADVISORY COMMITTEE

To: Pharmacology and Therapeutics Advisory Committee (PTAC)

From: Pharmac

Update on the paediatric cancer treatments review

Questions to PTAC Members for discussion

Please note that we do not intend for this discussion to be public knowledge and we are seeking your advice regarding the high level considerations that are important to understand during this review.

1. What are your views regarding the current inequities between paediatric patients with cancer and other patient groups?

2. What are you views regarding the risks of maintaining the current status quo regarding medicines access for paediatric cancer patients?

3. What are some of the key challenges that Pharmac could face with any change to the current funding of treatments for paediatric cancer patients and their family/whānau?

4. Do you consider that there are any reasons why assessing medicines for paediatric cancer patients would be difficult if we were to use the current available Pharmac processes? (eg Level of evidence, Medsafe approval)

    a. If so, why?

    b. If not, why not?

5. What are the key considerations that Pharmac should consider during implementation of any changes?

    a. Timing of changes (eg in the context of the Pharmac review and establishment of Health NZ and the Māori Health Authority)

    b. Engagement with external stakeholders

6. Are there any particular considerations that Pharmac should consider to address this?

    a. If so, what are these?

7. Who else should we be asking, in addition to those indicated in this paper, in order to develop a solution that works best for all?

    a. Who can we best engage with for Māori clinical and consumer/other advice in this area?

    b. What and who might be the most appropriate patient support and advocacy groups to engage with?

Purpose of Paper

Pharmac is in the early stages of reviewing how it funds paediatric pharmaceutical cancer treatments (PCTs) to ensure we are being transparent and fair compared with other patient groups.

This briefing provides you with background information on the review and our current progress and seeks your feedback on our early thinking.

Background

Paediatric PCTs are treated differently by Pharmac

Pharmac takes a different approach to paediatric PCTs than to how it funds treatments for other conditions.

This difference is due to rule 8.1b of the Pharmaceutical Schedule. Under this rule, District Health Board hospitals (DHBs) may give (and will be eligible to receive a subsidy for) any medicine for use within a paediatric oncology/haematology service for the treatment of cancer. This is known as the 'paediatric PCT pathway'.

Pharmac does not require medicines used under the paediatric PCT pathway to undergo the same decision making process that is required for Pharmaceutical Schedule listings or for other applications under Pharmac's Exceptional Circumstances Framework, such as Named Patient Pharmaceutical Assessment (NPPA) applications.

That means that, under the paediatric PCT pathway, paediatric oncologists and/or paediatric haematologists in New Zealand can give (within a DHB paediatric oncology/haematology service) any Pharmaceutical to children for the treatment of their cancer and it will be funded. This is regardless of the extent of very high need, evidence of benefit and whether or not it is cost-effective.

There is no separate budget allocation for paediatric PCTs. All Pharmaceutical treatments for paediatric patients with cancer, including those listed on the Pharmaceutical Schedule and those funded via the paediatric PCT mechanism, are funded from the Combined Pharmaceutical Budget (CPB).

The difference is historical

Before Pharmac's involvement, DHBs each undertook their own assessments and decision-making on cancer treatments, resulting in some inconsistency in the range of cancer medicines that could be accessed in different parts of the country. A key rationale for Pharmac's involvement was to support a more consistent approach to the funding of cancer medicines across the country.

When Pharmac was initially preparing to take on the role of PCTs, discussions with oncologists, haematologists and DHBs highlighted some complexities in relation to paediatric PCTs. These complexities made regular Pharmaceutical Schedule listings problematic in some cases.

Pharmac was not able to fully resolve these issues at the time, and in 2005 it was agreed that a separate funding mechanism specific to paediatric cancer treatments would be an appropriate solution. This resulted what is now Rule 8.1b of the Pharmaceutical Schedule – the "paediatric PCT pathway". It was intended at the time of creating Rule 8.1b that paediatric cancer treatments would remain outside of the usual funding pathways until a time that it needed to be reconsidered.

The different approach taken to the funding of paediatric PCTs has resulted in an inconsistency with the funding mechanisms for other treatments through the Schedule, including for other paediatric treatments and for adult cancer services.

Work has been underway to address the inconsistency since 2019

Aware of the inconsistency and the risk to the CPB with the advent of new technologies such as Chimeric Antigen Receptor (CAR) T-cell therapy, Pharmac began a review of the paediatric PCT pathway in early 2019. Analysis was undertaken of the numbers of patients, types of treatments and the cost of treatments being accessed through this pathway.

As noted above, the data showed that most medicines dispensed through the paediatric PCT pathway are already listed on the Pharmaceutical Schedule. Many of these are open listed currently and are likely available to paediatric patients with cancer. We note however, that there are some cancer treatments, currently listed on the schedule that may not be available to paediatric patients with cancer if there were a change to rule 8.1b.

Although the analysis did identify a trend towards expenditure increases, the work again highlighted the complexities of paediatric PCTs noted above. This, combined with the impact of COVID-19, resulted in the work being put on hold. In late 2020, work on the review recommenced.

The inconsistency was an aspect of a complaint to the Human Rights Commission

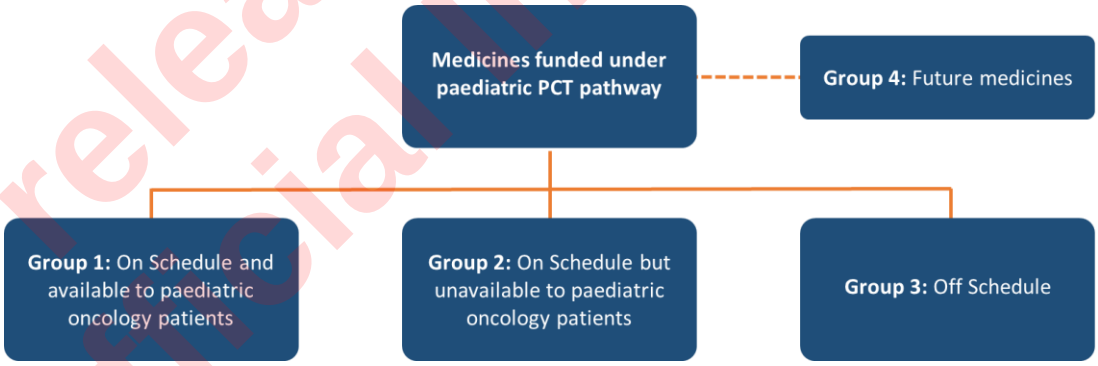
In May 2020, a complaint was lodged with the Human Rights Commission (HRC) that focused on the funding of nusinersen (Spinraza) for spinal muscular atrophy. The complaint highlighted the inconsistency between Pharmac's mechanism of funding for paediatric PCTs and treatments for other paediatric conditions.

Paediatric PCTs

Paediatric PCTs can be divided into four groups

These four groups of paediatric PCTs are outlined in **Figure 1** below:

Figure 1: Medicines funded via rule 8.1b of the Schedule



Each medicine group represented in **Figure 1** will require a different solution.

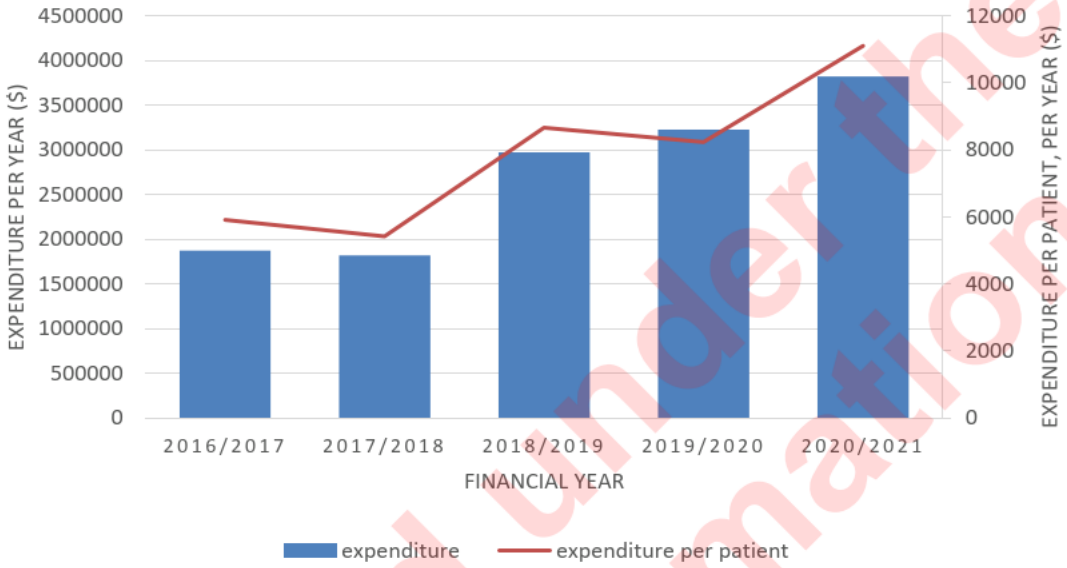
We understand that most treatments used by paediatric cancer patients are currently listed on the Pharmaceutical Schedule. However, it is unlikely that all paediatric cancer patients would meet the current eligibility criteria for all these medicines. We are in the process of obtaining cancer registration data from the Ministry of Health and mapping it to our medicine usage data to understand in what indications these medicines are used.

The expenditure on paediatric PCTs is relatively low but poses a future fiscal risk

We understand that approximately 150 patients access paediatric PCTs per year, with the majority being from Auckland.

The annual cost for all treatments in the 2019/2020 financial year was approximately $3 million. This cost has been increasing and is expected to increase further with the advent of novel, more expensive treatments (**Figure 2**).

Figure 2: Expenditure on paediatric PCTs from 2016/17 to 2020/21



The 2020/2021 financial year is likely to be even greater than that depicted, as this does not include data from the fourth quarter of the financial year. We note that this increase is**Error! Reference source not found.** driven by the use of newer products (eg blinatumomab). While we understand that DHBs have mechanisms in place to prioritise access and manage expenditure, Pharmac currently has no oversight of the decision-making processes that occur beyond the medicine that is used and the cost of each medicine.

Paediatric PCTs are complex and require a nuanced approach

The way that paediatric PCTs are funded has made any changes to this current access significantly complex. Some of the reasons for this complexity are listed below:

- Paediatric PCTs are used for a small number of patients
- Approximately one third of patients receive treatment as part of a clinical trial and funded access is required for treatments used as standard of care (non-investigational product)[1]
    - In many cases, these are not standard supplier-led clinical trials (with investigational product) and include drugs not listed on the Pharmaceutical Schedule
    - We note that accreditation and involvement in these trials is considered important for clinicians, and there are many people involved in maintaining this accreditation

[1] For other patient groups, anything associated with a clinical trial must be self-funded

- We note that there are additional benefits of access to treatment beyond the Pharmaceutical being applied for, and funded, by Pharmac (eg medicines provided free of charge)

- A minority of patients receive medicines not available to other patients via rule 8.1b.

- Rule 8.1b is considered to have provided good outcomes for this patient group. We understand that these good outcomes are not influenced by domicile or ethnicity. However, we acknowledge that the service in which these medicines are provided has a significant impact on these outcomes.

- Trying to make paediatric cancer treatment fit the adult oncology model, or that of other patient groups is difficult, because:

  - Timely access to treatments is required for these patients and there is a need to be able to move quickly to make these available.

  - Many of the medicines are not Medsafe approved and are unlikely to be.

  - The evidence supporting the use of the medicines in this patient group is less established.

  - Innate uncertainty regarding the use of these medicines.

  - It is widely considered that the current funding model does not adequately serve certain populations (eg paediatric or adult oncology).

- While certain cancers may be most common in children, these can occur in other age groups (eg adolescents and young adults). As with the rule 8.1b, in which funded access to treatments is dictated by the service within which the patient is being treated (usually driven by cancer type and age), changes to this would need to reflect the type of cancer rather than the age of the patient and therefore would likely need to include the adult and young adolescent age group.

Work underway

We have a set of principles that any change will need to satisfy.

These include:

- ensuring that all medicines currently used continue to be available to current and future patients

- ensuring that new paediatric PCTs are assessed in a way that is consistent with Pharmac's decision-making processes for other medicines

- prescribers being able to continue operating as similarly as possible to how they do now.

We have started to develop options

Using the principles outlined above, and our understanding of the medicines currently used via the paediatric PCT pathway, Pharmac has started to look at alternative options. This is being informed by an analysis of the latest available data on the treatments currently used through the paediatric PCT pathway.

We also need to consider options for how we could assess future Pharmaceutical Schedule funding applications for medicines used in paediatric cancers, as well as applications for medicines for individual paediatric patients with cancer (including in the context of a clinical trial).

There are other complexities that will need to be factored into our options development, including [redacted]

We have informed and sought advice from some groups

To date, Pharmac staff have informed and sought advice from the following:

- CaTSoP on 26 May 2021
- Ministry of Health (Te Aho o Te Kahu, Medical Oncology Working Group)

High level feedback to date has been:

- [redacted]

- Withheld under section 9(2)(f)(iv)
  Withheld under section 9(2)(f)(iv)
  Withheld

- The Ministry of Health has been informed of the review and supports the intent.

We plan to seek further input from:

- paediatric oncologists and haematologists in Auckland and Canterbury (the two national paediatric oncology centres)
- representatives of Māori and Pacific patients, communities, health professionals and health providers.
- Leukaemia & Blood Cancer New Zealand, CanTeen, and other patient support/advocacy groups
- Medsafe.

We would like your feedback

We are still at the early stages of this review. We want to take the necessary time needed to find the right solution, rather than rushing to implement what may eventuate to be an undesirable outcome.

Before we progress any further, we would like to hear PTAC Members' thoughts on the way that paediatric PCTs are funded and the impacts that any change to this could have. We acknowledge that there could be options available to Pharmac that have not been actively explored yet.

Once we have received the Committee's feedback, we will expand our engagement as our thinking develops. As well as the Committee's specific feedback, we would like to hear Members' views on who else we should be engaging with.

PHARMAC
TE PĀTAKA WHAIORANGA

Excerpt of Weekly media and issues update

For the week ending 30 July 2021

Upcoming issues and events

Subject	Timeframe	Details
Review into paediatric oncology treatments	Ongoing	The approach to funding paediatric oncology treatments is inconsistent with mechanisms for other treatments through the Combined Pharmaceutical Budget. We are weighing up options for how we review the paediatric oncology treatment pathway.

PHARMAC
TE PĀTAKA WHAIORANGA

Excerpt of Weekly media and issues update

For the week ending 6 August 2021

Upcoming issues and events

Subject	Timeframe	Details
Review into paediatric oncology treatments	Ongoing	The approach to funding paediatric oncology treatments is inconsistent with mechanisms for other treatments through the Combined Pharmaceutical Budget. We are weighing up options for how we review the paediatric oncology treatment pathway.

PHARMAC
TE PĀTAKA WHAIORANGA

CONFIDENTIAL – PLEASE DO NOT DISTRIBUTE

BRIEFING TO PAEDIATRIC ONCOLOGISTS AND HAEMATOLOGISTS

To:	**Paediatric oncologists and haematologists** Paediatric Blood & Cancer Centre service, Starship Children's Hospital, Child Haematology and Oncology Centre, Christchurch Hospital National Child Cancer Network (NCCN)
From:	Pharmaceutical Management Agency (Pharmac)
Date:	16 September 2021

Update on the paediatric cancer treatments review

Purpose of paper

Pharmac is in the early stages of reviewing how it funds certain paediatric pharmaceutical cancer treatments (paediatric PCTs). We are doing this because:

- We had always planned to revisit this mechanism for funding, which was not considered possible when Pharmac assumed the task of funding all cancer treatments
- there are questions that have been raised regarding the equitable treatment of this group and other patient groups
- we need to ensure that the way in which Pharmac funds treatments for all patient groups is sustainable in the future

This briefing provides you with background information on the reasons why we need to review this now and seeks your feedback on our early thinking.

Please note that prior to this meeting, we have received early informal feedback from our clinical advisors. We appreciate the complexities associated with the paediatric patient group and that any change could have considerable impact for them.

Before we progress any further, we would like to hear your thoughts on the current access to medicine for this group of patients, what this means for the treatment of these patients and how any change might impact the health outcomes for this patient group.

We would like your feedback on some of the considerations described below.

Background

Pharmac took over the management of cancer treatments *from DHBs*

Before 2005, DHBs each undertook their own assessments and decision-making for the provision of all cancer treatments. We understand that this caused some inconsistency in the range of cancer treatments that could be accessed in different parts of the country.

Following a review by the Ministry of Health and the New Zealand Cancer Treatments Working Party, the Government decided that a more consistent, nationwide approach to the funding of cancer treatments was needed. Pharmac was directed to take on the role of assessing and funding cancer treatments.

A new decision-making mechanism was developed for paediatric PCTs in 2005

When Pharmac was initially preparing to take on the role of assessing and funding cancer treatments, discussions with oncologists, haematologists and DHBs highlighted some complexities in relation to the treatment of paediatric cancer patients. This included:

- 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;
- 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

At the time, Pharmac was not routinely listing unapproved treatments in the Schedule; in fact, it was very rare for us to do so. These complexities made regular Pharmaceutical Schedule listings problematic in some cases.

Pharmac was not able to fully resolve these issues. It was agreed that a separate funding mechanism specific to paediatric cancer treatment would be an appropriate solution.

This resulted in what is now rule 8.1b of the Pharmaceutical Schedule. Under this rule, DHBs may give (and will be eligible to receive a subsidy for) any medicine for use within a paediatric oncology/haematology service for the treatment of cancer. This is known as the 'paediatric PCT pathway'. This is regardless of any Pharmac oversight of extent of health need, evidence of benefit and whether or not it is cost-effective. Rule 8.1b means that Pharmac does not require paediatric PCTs to undergo the same decision making process that is required for Pharmaceutical Schedule listings or for other applications under Pharmac's Exceptional Circumstances Framework.

It was intended at the time of creating Rule 8.1b that paediatric cancer treatments not listed in the Pharmaceutical Schedule would remain outside of the usual funding pathways until a time that it needed to be reconsidered.

There is no separate budget allocation for paediatric PCTs. All pharmaceutical treatments for paediatric (child/some adolescent) patients with cancer, including those listed on the Pharmaceutical Schedule and those funded via the paediatric PCT mechanism, are funded from the Combined Pharmaceutical Budget (CPB).

This rule has created an inconsistency between Paediatric PCTs and other treatments

Rule 8.1b has resulted in an inconsistency with the funding mechanisms for other treatments listed on the Pharmaceutical Schedule, including treatments for other paediatric populations, treatments for adult cancer patients and treatments for all other patients.

Additionally, continuation of the current funding approach may present a risk to the CPB if cost increases cannot be appropriately forecast or contained, particularly considering the increasing development of expensive, precision treatments for childhood cancers. Without Pharmac's oversight, this presents a risk that increased expenditure on paediatric PCTs may result in less funding available for other patient groups and conditions. This could perpetuate further inequities between child cancer patients and other patient groups.

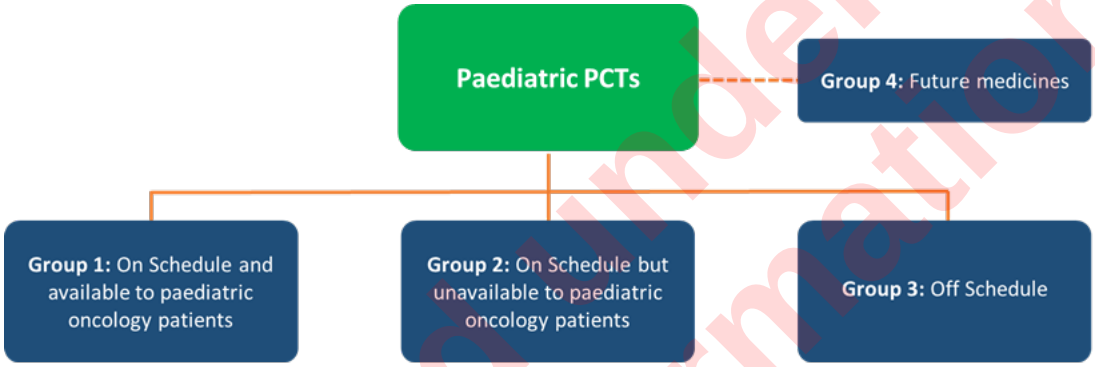
Pharmac's review of paediatric cancer treatments

Pharmac first started reviewing paediatric cancer treatments in 2019 and re-commenced this review in early 2021.

Paediatric PCTs can be divided into four groups for simplicity

These are outlined in **Figure 1**:

Figure 1: Paediatric PCTs funded by Pharmac



We consider that each group of treatments represented in **Figure 1** would need to be treated differently.

We understand that many treatments used by paediatric cancer patients are currently listed on the Pharmaceutical Schedule. However, it is unlikely that all paediatric cancer patients would meet the current eligibility criteria for all of these treatments. We are in the process of obtaining cancer registration data from the Ministry of Health and mapping it to our medicine usage data to understand in what indications these treatments are used. We hope to be able to sense check some of this with you as this work progresses.

The cost impact of paediatric PCTs is relatively low but poses a future risk

Our data indicates that approximately 330 paediatric patients with cancer are dispensed oncology treatments each year and that the approximate ethnic makeup (based on primary ethnicity) of these patients in the 2020/2021 financial year (ending 30 June) was:

- 44% New Zealand European
- 29% Māori
- 14% Asian
- 10% Pacific peoples
- 3% Middle Eastern, African, and Latin American
- 11% Other

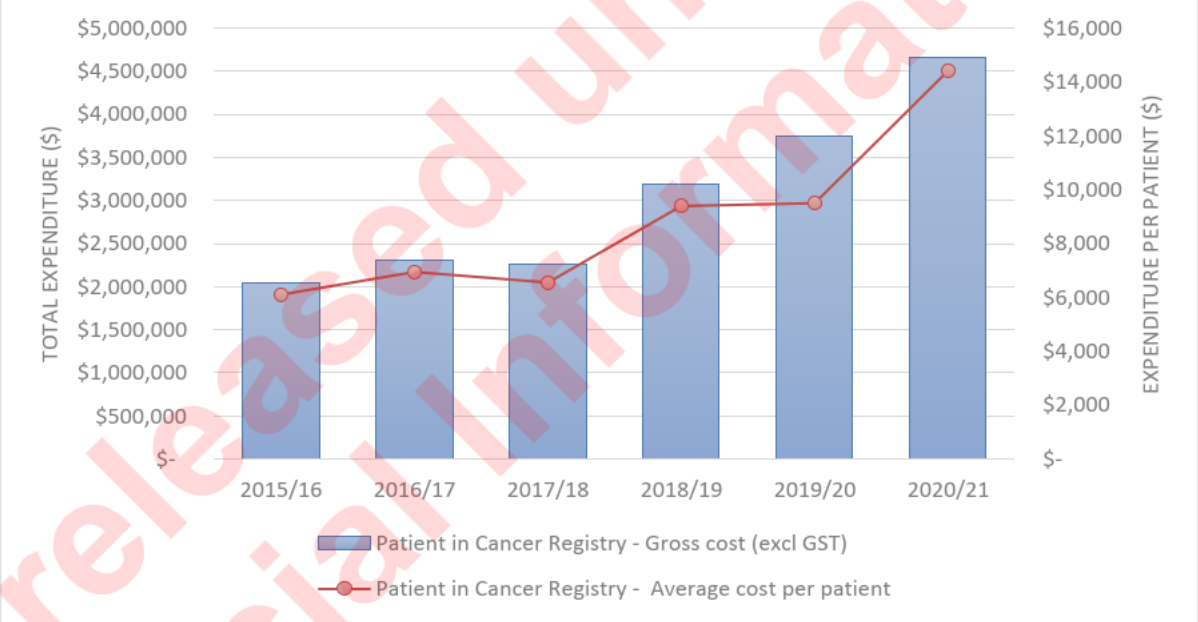
We estimate from the data we have available that approximately 40 paediatric patients access cancer treatments through the funding pathway created by rule 8.1b each year.

Our data indicates that annual expenditure on treatments for children with cancer in the 2019/2020 financial year was approximately $3.7 million. This expenditure has been increasing since 2017/2018 and is expected to increase further with the advent of novel, more expensive treatments (**Figure 2**).

Please note that **Figure 2** does not include the expenditure for new patients that have presented in the 2021 calendar year (due to a delay in the cancer registry data being available). Therefore, the total expenditure is likely to be greater than the $4.7 million depicted. This is reflected in the average cost per patient, which has increased substantially since the 2017/28 financial year. We consider that this increase is**Error! Reference source not found.** likely driven by the use of more expensive newer products for small numbers of patients (eg. blinatumomab).

While we understand and appreciate that the service has mechanisms in place to prioritise access to treatment and manage expenditure, Pharmac currently has no oversight of the decision-making processes that occur beyond the medicine that is used, the indication, and the cost of each medicine.

Figure 2: Expenditure on paediatric PCTs from 2015/16 – 2020/21



Paediatric PCTs are complex and require a nuanced approach

The current paediatric PCT funding pathway is associated with a number of complexities that could make alteration to the current system challenging. We have briefly indicated our understanding of some of these complexities below, and would like to discuss these and any further complexities with you:

- At this time, we do not have a good understanding of the ways in which the treatments that are claimed via rule 8.1b are being used.

- Any changes to the current approach could result in reduced access to paediatric PCTs.

- Approximately one third of patients receive treatment as part of a clinical trial/formal research protocol and funded access is required for treatments used as standard of care (non-investigational product)
    - in many cases, these are not standard supplier-led clinical trials (with investigational product) and include treatments not listed on the Pharmaceutical Schedule
    - accreditation and involvement in these trials is important for clinicians as it helps support access to the latest treatment protocols, and there are many people involved in maintaining this accreditation
    - the clinical trials involve surrogate outcomes and incur frequent revisions to the trial protocols
    - there may be additional benefits of access to treatment beyond the funded pharmaceutical (eg treatments provided free of charge).
- A small proportion of children with cancer receive treatments through rule 8.1b but this patient group would be vulnerable to a change in medicine availability.
- Rule 8.1b is considered to have enabled good outcomes for this patient group. We understand that these good outcomes are not influenced by DHB of domicile or ethnicity.
- While certain cancers may be most common in children, these same cancers can occur in other age groups (eg adolescents and young adults). Therefore, any changes would likely need to include the adult and young adolescent age group to ensure that the changes are equitable.

We can appreciate that trying to make paediatric cancer treatments fit the adult oncology model, or that of other patient groups could be challenging, because:

- timely access to treatment is required for these patients to be able to receive treatment as part of trial protocols or otherwise, noting that these protocols are frequently revised
- many of the treatments are not Medsafe approved and are unlikely to be
- the evidence supporting the use of treatments in this patient group may be less established, and often emerge once investigators have moved onto a new research protocol

We have developed a set of principles that we consider an alternative to the status quo would need to meet

These principles would ensure that an alternative solution could respond to the complexities associated with paediatric PCTs. These include:

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

Potential options

Withheld under section 9(2)(f)(iv)

Withheld under section 9(2)(f)(iv)
Withheld under section 9(2)(f)(iv)
Withheld under section 9(2)(f)(iv)

Withheld under section 9(2)(f)(iv)

Withheld under section 9(2)(f)(iv)

Withheld under section 9(2)(f)(iv)

Withheld under section 9(2)(f)(iv)
Withheld under section 9(2)(f)(iv)
Withheld

Withheld under section 9(2)(f)(iv)
Withheld under section 9(2)(f)(iv)

Withheld under section 9(2)(f)(iv)
Withheld under section 9(2)(f)(iv)

We are interested in whether you consider any, or a combination of these options to be reasonable, workable alternatives to the status quo. In addition, we would like to understand if there are any other potential options that have not been included above.

Considerations for discussion

We would like to understand

- how many patients are treated within the paediatric cancer service in New Zealand per year in Christchurch and Auckland.
- how this care is provided, as well as the relationship with other DHBs with respect to the ongoing treatment of these patients.
- in more detail, how decisions for the use of rule 8.1b are prioritised and made currently for children/adolescents with cancer.

As an interim option to improve our understanding, we would like to know if you would be comfortable providing more information to Pharmac when rule 8.1b is used, specifically in relation to the records of the multidisciplinary meetings.

We seek your feedback on:

- some of our thinking around potential alternative options to the status quo, as well as your insight into other potential alternatives that we could consider as part of this review.
- the potential impact to patients and clinicians regarding any of these potential changes.
- who else we should be seeking input and feedback from at this early stage.

PHARMAC
TE PĀTAKA WHAIORANGA

CONFIDENTIAL – PLEASE DO NOT DISTRIBUTE

BRIEFING TO PAEDIATRIC ONCOLOGISTS AND HAEMATOLOGISTS

To:	**Paediatric oncologists and haematologists** Paediatric Blood & Cancer Centre service, Starship Children's Hospital, Child Haematology and Oncology Centre, Christchurch Hospital National Child Cancer Network (NCCN)
From:	Pharmaceutical Management Agency (Pharmac)
Date:	Updated post-meeting on 21 September 2021

Update on the paediatric cancer treatments review

Purpose of paper

Pharmac is in the early stages of reviewing how it funds certain paediatric pharmaceutical cancer treatments (paediatric PCTs). We are doing this because:

- we had always planned to revisit this mechanism for funding, which was not considered possible when Pharmac assumed the task of funding all cancer treatments
- there are questions that have been raised regarding the equitable treatment of this group and other patient groups
- we need to ensure that the way in which Pharmac funds treatments for all patient groups is sustainable in the future

This briefing provides you with background information on the reasons why we need to review this now and seeks your feedback on our early thinking.

Please note that prior to this meeting, we have received early informal feedback from our clinical advisors. We appreciate the complexities associated with the paediatric patient group and that any change could have considerable impact for them.

Before we progress any further, we would like to hear your thoughts on the current access to medicine for this group of patients, what this means for the treatment of these patients and how any change might impact the health outcomes for this patient group.

We would like your feedback on some of the considerations described below.

This briefing paper has been updated after the 21 September 2021 meeting between the Paediatric Blood Cancer Centre Service at Starship Children's Hospital, the Child Haematology and Oncology Centre at Christchurch Hospital, the National Child Cancer Network and Pharmac. The updates clarify some aspects of the information presented and support wider circulation of this briefing with all relevant paediatric oncologists and haematologists in these services.

Background

Pharmac took over the management of cancer treatments *from DHBs*

Before 2005, DHBs each undertook their own assessments and decision-making for the provision of all cancer treatments. We understand that this caused some inconsistency in the range of cancer treatments that could be accessed in different parts of the country.

Following a review by the Ministry of Health and the New Zealand Cancer Treatments Working Party, the Government decided that a more consistent, nationwide approach to the funding of cancer treatments was needed. Pharmac was directed to take on the role of assessing and funding cancer treatments.

A new decision-making mechanism was developed for paediatric PCTs in 2005

When Pharmac was initially preparing to take on the role of assessing and funding cancer treatments, discussions with oncologists, haematologists and DHBs highlighted some complexities in relation to the treatment of paediatric cancer patients. This included:

- 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;
- 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

At the time, Pharmac was not routinely listing unapproved treatments in the Schedule; in fact, it was very rare for us to do so. These complexities made regular Pharmaceutical Schedule listings problematic in some cases.

Pharmac was not able to fully resolve these issues. Following conversations between Pharmac and DHBs, it was agreed that a separate funding mechanism specific to paediatric cancer treatment would be an appropriate solution.

This resulted in what is now rule 8.1b of the Pharmaceutical Schedule. Under this rule, DHBs may give (and will be eligible to receive a subsidy for) any medicine for use within a paediatric oncology/haematology service for the treatment of cancer. This is known as the 'paediatric PCT pathway'. This is regardless of any Pharmac oversight of extent of health need, evidence of benefit and whether or not it is cost-effective. Rule 8.1b means that Pharmac does not require paediatric PCTs to undergo the same decision making process that is required for Pharmaceutical Schedule listings or for other applications under Pharmac's Exceptional Circumstances Framework.

It was intended at the time of creating Rule 8.1b that paediatric cancer treatments not listed in the Pharmaceutical Schedule would remain outside of the usual funding pathways until a time that it needed to be reconsidered.

There is no separate budget allocation for paediatric PCTs. All pharmaceutical treatments for paediatric (child/some adolescent) patients with cancer, including those listed on the Pharmaceutical Schedule and those funded via the paediatric PCT mechanism, are funded from the Combined Pharmaceutical Budget (CPB).

This rule has created an inconsistency between Paediatric PCTs and other treatments

Rule 8.1b has resulted in an inconsistency with the funding mechanisms for other treatments listed on the Pharmaceutical Schedule, including treatments for other paediatric populations, treatments for adult cancer patients and treatments for all other patients.

Additionally, continuation of the current funding approach may present a risk to the CPB if cost increases cannot be appropriately forecast or contained, particularly considering the increasing development of expensive, precision treatments for childhood cancers. Without Pharmac's oversight, this presents a risk that increased expenditure on paediatric PCTs may result in less funding available for other patient groups and conditions. This could perpetuate further inequities between child cancer patients and other patient groups.

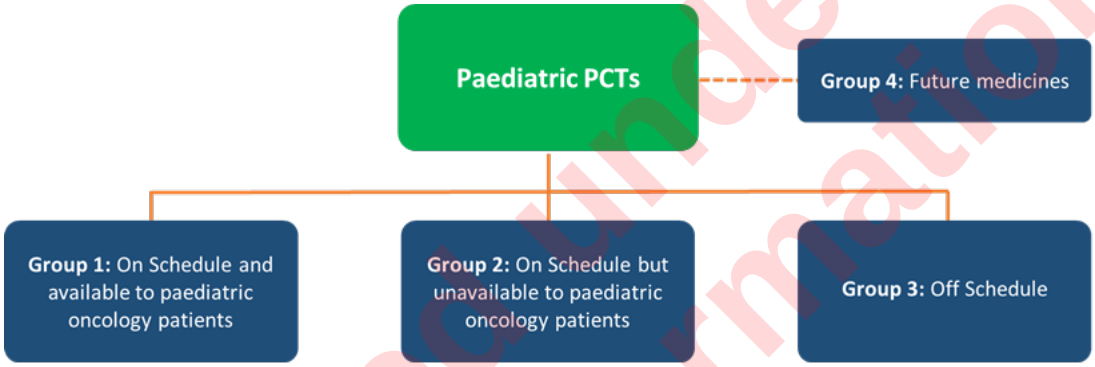
Pharmac's review of paediatric cancer treatments

Pharmac first started reviewing paediatric cancer treatments in 2019 and re-commenced this review in early 2021.

Paediatric PCTs can be divided into four groups for simplicity

These are outlined in **Figure 1**:

Figure 1: Paediatric PCTs funded by Pharmac



We consider that each group of treatments represented in **Figure 1** would need to be treated differently.

We understand that many treatments used by paediatric cancer patients are currently listed on the Pharmaceutical Schedule. However, it is unlikely that all paediatric cancer patients would meet the current eligibility criteria for all of these treatments. We are in the process of obtaining cancer registration data from the Ministry of Health and mapping it to our medicine usage data to understand in what indications these treatments are used. We hope to be able to sense check some of this with you as this work progresses.

The cost impact of paediatric PCTs is relatively low but poses a future risk

Our data indicates that approximately 330 paediatric patients with cancer are dispensed oncology treatments each year and that the approximate ethnic makeup (based on primary ethnicity) of these patients in the 2020/2021 financial year (ending 30 June) was:

- 44% New Zealand European
- 29% Māori
- 14% Asian
- 10% Pacific peoples
- 3% Middle Eastern, African, and Latin American
- 11% Other

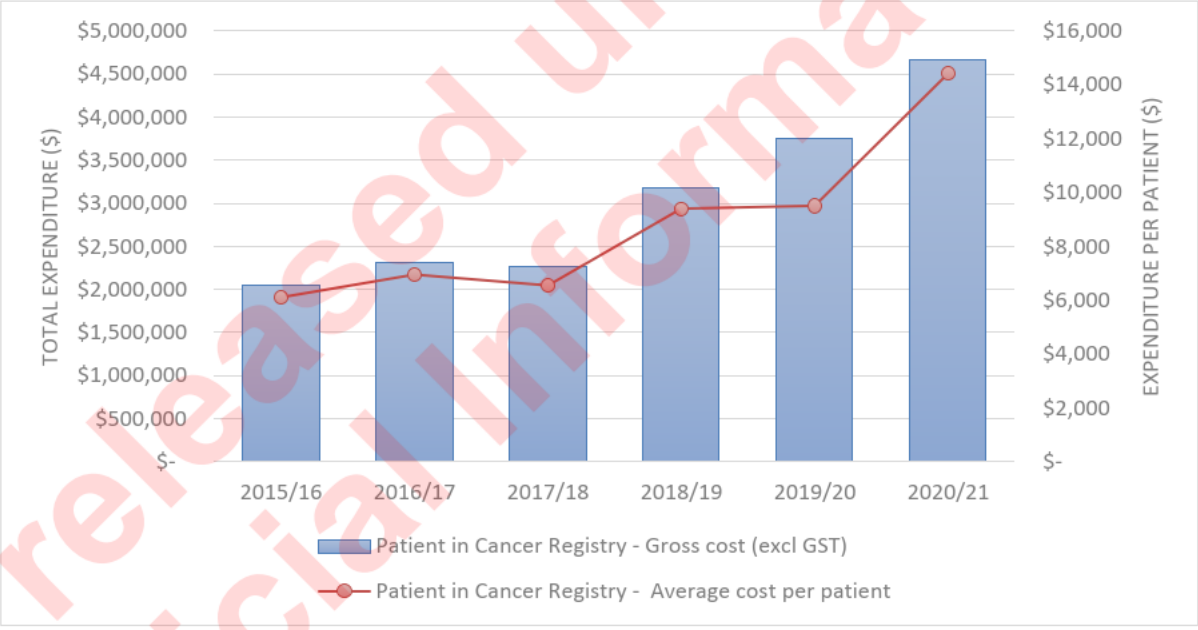
We estimate from the data we have available that approximately 40 paediatric patients access cancer treatments through the funding pathway created by rule 8.1b each year.

Our data indicates that annual expenditure on treatments for people with cancer (including those treatments accessed via Pharmaceutical Schedule listing) born after January 1996 in the 2019/2020 financial year was approximately $3.7 million. This expenditure has been increasing since 2017/2018 and is expected to increase further with the advent of novel, more expensive treatments (**Figure 2**).

Please note that **Figure 2** does not include the expenditure for new patients that have presented in the 2021 calendar year (due to a delay in the cancer registry data being available). Therefore, the total gross expenditure is likely to be greater than the $4.7 million depicted. This is reflected in the average cost per patient, which has increased substantially since the 2017/28 financial year. We consider that this increase is**Error! Reference source not found.** likely driven by the use of more expensive newer products for small numbers of patients (eg. blinatumomab).

While we understand and appreciate that the service has mechanisms in place to prioritise access to treatment and manage expenditure, Pharmac currently has no oversight of the decision-making processes that occur beyond the medicine that is used, the indication, and the cost of each medicine.

Figure 2: Expenditure on paediatric PCTs from 2015/16 – 2020/21



Paediatric PCTs are complex and require a nuanced approach

The current paediatric PCT funding pathway is associated with a number of complexities that could make alteration to the current system challenging. We have briefly indicated our understanding of some of these complexities below, and would like to discuss these and any further complexities with you:

- At this time, we do not have a good understanding of the ways in which the treatments that are claimed via rule 8.1b are being used. Paediatric treatment protocols are often complex and involve more than one medicine being administered to a patient.

- Any changes to the current approach could result in reduced or delayed access to paediatric PCTs.
    - Patients could be more likely to seek private healthcare – this would only benefit those who have the means to do so. There is no private paediatric oncology service in New Zealand.
        - This may affect outcomes; one of the reasons why there are currently equitable outcomes is that patients are only treated in two treatment centres with dedicated paediatric oncologists.

- Approximately one third of patients receive treatment as part of a clinical trial/formal research protocol and funded access is required for treatments used as standard of care (non-investigational product).
    - In many cases, these are not standard supplier-led clinical trials (with investigational product) and include treatments not listed on the Pharmaceutical Schedule
        - The ability to participate in many clinical trials requires access to certain medicines, many of which may be accessed through rule 8.1b.
    - Large quantities of medicines free of charge have been accessed for patients through clinical trials.
        - Access to data that reflects the use of publicly funded treatments means that the value of access to medicines used in clinical trials is not known and therefore the potential return on investment is uncertain.
    - Clinical trials enable patients to access services, including diagnostics, that are not currently available in New Zealand. It also enables access to detailed treatment protocols, which enable more effective treatment of patients.
    - Participation means New Zealand-based clinicians are forced to undergo external peer review of their clinical activity. This benefits clinical practice – literature proves that units which are clinical trial-based have superior clinical performance in terms of quality of care and quantity of survival.
        - Accreditation and involvement in these trials is important for clinicians as it helps support access to the latest treatment protocols, and there are many people involved in maintaining this accreditation.

- A small proportion of children with cancer receive treatments through rule 8.1b but this patient group would be vulnerable to a change in medicine availability.

- It would be difficult to exclude the use of medicines in paediatric oncology based on a lack of published evidence. Paediatric oncology trials are designed to build off each other and allow clinicians to exchange information. They are an ongoing interactive process, and it is difficult to tease out what exact components of a trial benefit patients.
    - The clinical trials involve surrogate outcomes and incur frequent revisions to the trial protocols. Successive trials build on the anticipation of results and while outcomes are eventually published a new protocol is already in place.

- Rule 8.1b is considered to have enabled good outcomes for this patient group – an approximately 84% five-year survival rate, which is not influenced by DHB, domicile or ethnicity.[1]
    - New Zealand's five-year child cancer survival rate is on par with other OECD countries.

- While certain cancers may be most common in children, these same cancers can occur in other age groups (eg adolescents and young adults [AYAs]).

    - Currently many AYAs do not have access to the same treatment as paediatric patients as they are treated by adult oncology services despite similar cancer type and morphology. Therefore, any changes would likely need to include consideration of the AYA group to ensure that any changes are equitable.

We can appreciate that trying to make paediatric cancer treatments fit the adult oncology model, or that of other patient groups could be challenging, because:

- timely access to treatment is required for these patients to be able to receive treatment as part of trial protocols or otherwise, noting that these protocols are frequently revised

- many of the treatments are not Medsafe approved and are unlikely to be

- the evidence supporting the use of treatments in this patient group may be less established, and often emerge once investigators have moved onto a new research protocol.

We have developed a set of principles that we consider an alternative to the status quo would need to meet

These principles would ensure that an alternative solution could respond to the complexities associated with paediatric PCTs. These include:

- ensuring that all treatments currently used continue to be available to current and future patients

- ensuring that new paediatric PCTs are assessed in a way that is consistent with Pharmac's decision-making processes for other treatments

- ensuring that prescribers being able to continue operating as similarly as possible to how they do now.

Potential options

Withheld under section 9(2)(f)(iv)

Withheld under section 9(2)(f)(iv)

Withheld under section 9(2)(f)(iv)

Withheld under section 9(2)(f)(iv)

Withheld under section 9(2)(f)(iv)

Withheld under section 9(2)(f)(iv)

[1] https://childcancernetwork.org.nz/wp-content/uploads/2017/12/Childhood-Cancer-Survival-in-New-Zealand-2005-2014.pdf

Withheld under section 9(2)(f)(iv)

Withheld under section 9(2)(f)(iv)
Withheld under section 9(2)(f)(iv)
Withheld

Withheld under section 9(2)(f)(iv)
Withheld under section 9(2)(f)(iv)

Withheld under section 9(2)(f)(iv)
Withheld under section 9(2)(f)(iv)

We are interested in whether you consider any, or a combination of these options to be reasonable, workable alternatives to the status quo. In addition, we would like to understand if there are any other potential options that have not been included above.

Excerpt of Pharmac weekly key messages

Updated Thursday 7 October 2021

Paediatric Oncology review

Background

Media have asked for an update on the Paediatric Oncology review, following Shane Reti's Private Members Bill being pulled for consideration.

Communications approach

Reactive messages prepared. Lisa Williams will be Pharmac's spokesperson on this issue.

Main message

- PHARMAC is reviewing how we fund paediatric cancer treatments because its inconsistent with how we fund other medicines. At this stage no decisions have been made.

Core messages

- We are reviewing rule 8.1b of the Pharmaceutical Schedule. This is an exemption which allows DHBs hospital to give (and receive a subsidy for) any paediatric oncology or haematology medicine when treating paediatric oncology patients.
- Medicines being used for paediatric cancer treatments now, will continue to be available for our children and young people. Any treatments currently in use will stay in use.
- While we believe the current paediatric oncology exception does not constitute unlawful discrimination, New Zealanders have asked why these medicines are funded differently. We are reviewing the exception to be transparent and fair.
- We have not made any decisions about what would change or if even if there would be any changes.
- We hope to work out a way to fairly and consistently deal with paediatric oncology treatments compared with other medicines for other conditions.
- As we work through potential options, we will consult widely and seek feedback from the public.