TE AHO O TE KAHU
CANCER CONTROL AGENCY

Te rere o te toto
Understanding blood cancer medicine availability in Aotearoa New Zealand

Summary report

Citation: Te Aho o Te Kahu. 2024. *Te rere o te toto – Understanding blood cancer medicine availability in Aotearoa New Zealand: Summary report*. Wellington: Te Aho o Te Kahu.

Published in October 2024 by Te Aho o Te Kahu – Cancer Control Agency
PO Box 5013, Wellington 6140, New Zealand

ISBN 978-1-7385872-1-6 (online)
TP0015

TE AHO O TE KAHU CANCER CONTROL AGENCY

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HE KUPU TAKAMUA
FOREWORD

Tēnā koutou,

Every year in Aotearoa New Zealand, around 2,800 people are diagnosed with a blood cancer. There are no known prevention or screening interventions for blood cancers. For the people diagnosed, cancer medicines are often the principal treatment option. It is incredibly important to all people with cancer, their whānau, and their cancer treatment teams that the most appropriate treatment is available to them, when and where they need it.

For a number of years, there has been concern that Australia has more cancer medicines available than Aotearoa New Zealand. The report *Understanding the Gap: an analysis of the availability of cancer medicines in Aotearoa* was written in 2022 to provide clarity as to the extent and materiality of the difference in medicine availability between Australia and Aotearoa New Zealand. Only medicines that met a minimum threshold of clinical benefit (based on an internationally recognised tool) were identified as a gap in the report.

At the time of the 2022 report, it was not possible to assess the magnitude of clinical benefit for blood cancer medicines funded in Australia but not in Aotearoa New Zealand. Te Aho o Te Kahu made the commitment to complete that part of the analysis as soon as possible. We are very pleased to now deliver on that commitment and complete this work.

The information in this report will make clear how blood cancer medicine availability in Aotearoa New Zealand is different from Australia, a country we are routinely compared with. We hope the report will contribute further to the understanding of the availability of cancer medicines in Aotearoa New Zealand and that it will be a useful reference for the health system. The insights gained from this report can add to the discourse about access to medicines in this country and be considered alongside the many other sources of evidence and advice that inform decisions about medicines funding.

Cancer medicines are an important part of providing quality cancer care, but they are just one of a broad range of approaches needed to combat cancer. Coordinated efforts across the entire cancer continuum are required to ensure the system functions as efficiently and equitably as possible to deliver the best health outcomes. This includes focusing on prevention activities, identifying and diagnosing people with cancer early (including through screening), and ensuring there is adequate workforce and infrastructure to support high-quality care (including surgery and radiation therapy) and quality-of-life supports (including palliative and end-of-life care). The full health benefits of cancer medicines can only be realised if all parts of the cancer care continuum are working well and adequately resourced.

People with cancer, and their whānau, must always remain at the centre of any conversation about cancer treatments. While this report is technical, it has been written with those people in mind.

I would like to thank all those who provided their expertise to support this report, including the staff at Te Aho o Te Kahu and the wider project team of clinical pharmacists and haematologists who are at the front line of care delivery. The release of this report brings to completion over three years of work for this group.

It is my hope that this report will provide additional clarity and insights to all those invested in ensuring there are fewer cancers, better survival, and equity for all.

Mauri ora

Rami Rahal
Chief Executive and National Director of Cancer Control
Te Aho o Te Kahu | Cancer Control Agency

NGĀ IHIRANGI
CONTENTS

HE KUPU TAKAMUA | FOREWORD iii

TE RERE O TE TOTO: UNDERSTANDING BLOOD CANCER MEDICINE AVAILABILITY IN AOTEAROA – SUMMARY REPORT 1

- Cancer has a significant impact on the health of New Zealanders 1
- Cancer medicines are a critical part of cancer treatment 2
- Cancer medicine funding decisions are complex 2
- Why compare which blood cancer medicines are funded in Australia and Aotearoa New Zealand? 3
- The findings of the 2022 report 3
- How the 2024 report differs to the 2022 report 4
- What did the 2024 report involve? 4
- What did we find? 8
- What are the key strengths and limitations of this analysis? 9
- Key things to consider when interpreting the findings of this report 10

HE KUPU WHAKAKAPI | CONCLUSION 12

NGĀ ĀPITIHANGA | APPENDICES 13

- Appendix 1: Summary of medicine availability gaps by type of cancer and ESMO-MCBS:H score 13
- Appendix 2: Medicine-indication pairs funded in Australia but not in Aotearoa New Zealand with a substantial magnitude of clinical benefit 14
- Appendix 3: Medicine-indication pairs funded in Australia but not in Aotearoa New Zealand that were determined to not have a substantial magnitude of clinical benefit 17
- Appendix 4: Key sources of information 23

List of figures

Figure 1: ESMO-MCBS:H possible scores 7

Figure 2: Summary of results in blood medicine availability between Australia and Aotearoa New Zealand on 1 January 2024 8

TE RERE O TE TOTO: UNDERSTANDING BLOOD CANCER MEDICINE AVAILABILITY IN AOTEAROA – SUMMARY REPORT

Cancer has a significant impact on the health of New Zealanders

Cancer is the leading cause of death in Aotearoa New Zealand and is a major contributor to loss of health. Each year, approximately 27,000 people are diagnosed with cancer and around 9,000 people die of cancer in Aotearoa New Zealand. Ninety percent of those people diagnosed with cancer, or dying from cancer, have a solid tumour cancer. The remaining 10% have a blood cancer. The most diagnosed solid tumour cancers are breast, lung, prostate and colorectal cancers. The most commonly diagnosed blood cancers are non-Hodgkin's lymphoma, leukaemia, and myeloma.

Cancer does not impact everyone equally. The burden of cancer disproportionally affects Māori, who are diagnosed at greater rates, often at younger ages, with more severe disease and have poorer survival outcomes. Other inequities are also evident, with high rates of cancer incidence and death experienced by Pacific peoples, disabled people, and New Zealanders living in areas of high deprivation. These inequities in cancer are unjust and unfair and are the result of many factors.

We know that timely access to cancer medicines is a central part of cancer treatment for many New Zealanders. We also know that the public funding of medicines, including cancer medicines, is complex due to the considerable number of medicines available, the increasing cost of medicines and limited budgets. Although these factors are not unique to our country, it is generally established that Aotearoa New Zealand has access to fewer cancer medicines than countries we like to compare ourselves to, including Australia, the United Kingdom and Canada.

Cancer medicines are a critical part of cancer treatment

Access to appropriate and effective cancer medicines is central to high-quality cancer care. Many people with cancer will have cancer medicines as part of their treatment plan. For those with blood cancer, medicines are the primary, and often the only, treatment option. Some people with blood cancers may also be able to have a stem cell transplant, receive radiation therapy or need surgery. However, people with solid tumours are more likely to receive medicines in combination with surgical interventions and radiation therapy.

Cancer medicines are commonly administered to a person as a series of infusions in a hospital setting, or orally as tablets/capsules that may be taken at home, in primary care or in a hospital setting. Medicines may be used with an aim of curing cancer, prolonging survival, or improving quality of life.

Cancer medicine funding decisions are complex

All countries must assess the value of new medicines and choose which medicines to fund. The way this is achieved varies between countries, but the assessment and decision-making process is always complex. There are benefits and drawbacks to all approaches.

Decisions about which medicines are publicly funded in Aotearoa New Zealand are made by Pharmac. Pharmac is an independent Crown entity tasked with publicly funding medicines that secure the best health outcomes that can reasonably be achieved within the fixed pharmaceutical budget it is allocated by the Government.

Budget constraints, combined with the high cost of many medicines, mean Aotearoa New Zealand, like other countries, cannot fund every medicine on the market. This requires prioritised decisions about the medicines that can be funded in Aotearoa New Zealand.

Pharmac uses a framework called the Factors for Consideration to evaluate the benefit of a proposed new medicine and how this compares to other proposed new medicines. The Factors for Consideration include:

- the health need of the patient population
- the health/clinical benefits of the new medicine compared to what is already available
- the cost implication of funding this new medicine, including potential savings
- how suitable the new medicine is compared to what patients receive now.

Using the Factors for Consideration, Pharmac creates a list of medicines in priority order that it would fund if/when budget is available. This is the Options for Investment (OFI) list. Additional budget to fund new medicines primarily arises due to:

- savings Pharmac achieves in payment for medicines it already funds
- additional budget provided by the Government.

As of October 2024, there are 132 medicines on the OFI list that Pharmac would fund if it had available budget. Fifty are for the treatment of cancer generally, with 14 specifically for blood cancers.

Why compare which blood cancer medicines are funded in Australia and Aotearoa New Zealand?

It is generally established that Aotearoa New Zealand has access to fewer cancer medicines than countries we like to compare ourselves to, including Australia, the United Kingdom and Canada.

In 2022 Te Aho o Te Kahu published *Mārama ana ki te Āputa: he tātari i te wāteatanga o ngā rongoā mate pukupuku i Aotearoa | Understanding the Gap: an analysis of the availability of cancer medicines in Aotearoa* (the 2022 report),[1] which confirmed that more cancer medicines were publicly funded in Australia than in Aotearoa New Zealand (medicine 'gaps'), and some of the gaps were associated with substantial clinical benefit. The 2022 report was designed to improve understanding of the difference in availability of publicly funded cancer medicines between Australia and Aotearoa New Zealand and the clinical benefit those medicines would provide if they were available in Aotearoa New Zealand.

The findings of the 2022 report

The 2022 report found that there were 72 individual cancer medicines available in Australia but not in Aotearoa New Zealand.[2] This included medicines that were available in Aotearoa New Zealand but for the treatment of a different cancer than it is used for in Australia. The 72 individual medicines translated to 126 different treatment uses, called indications, 98 of which were for solid tumour cancers and 28 for blood cancers. This decreased to 88 regimens for solid tumour cancers and 26 for blood cancers when medicines used in combination were taken into account (that is, when two or more medicines are used at the same time as part of one treatment).

The 2022 report also assessed the clinical benefit the medicine gaps would provide if they were funded in Aotearoa New Zealand. This was achieved using a validated tool developed by the European Society for Medical Oncology (ESMO) called the Magnitude

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1 **teaho.govt.nz/publications/cancer-medicines**

2 In the 2022 and 2024 reports, 'available medicines' refers to publicly funded medicines. For Aotearoa New Zealand, this means medicines funded by Pharmac.

of Clinical Benefit Scale (ESMO-MCBS).[3] At the time the analysis was conducted the tool was only available to assess the clinical benefit of solid tumour cancer medicines.

Three of the identified gaps were likely to be associated with substantial clinical benefit in a curative clinical setting, and 17 additional gaps were likely to be associated with substantial clinical benefit in a non-curative setting. Since the publication of the 2022 report and the 1 January 2024, five of the identified 20 have been funded by Pharmac and are publicly available in Aotearoa New Zealand.

How the 2024 report differs to the 2022 report

The 2022 report did not comment on the magnitude of clinical benefit for blood cancer medicines that were available in Australia but not in Aotearoa New Zealand. This was because the ESMO-MCBS was not validated for blood cancers at that time.

In 2023, ESMO released a new version of the ESMO-MCBS called the ESMO-MCBS:H ('H' for haematological or blood cancers), which can be used to assess the clinical value of blood cancer medicines. This version of the tool allowed Te Aho o Te Kahu to determine the magnitude of clinical benefit for blood cancer medicines and complete the picture of medicine availability differences between Australia and Aotearoa New Zealand.

While a very similar methodology was used for both the 2022 and 2024 reports, it should be noted that the calculation of magnitude of clinical benefit scores did differ for some medicines in each analysis. The magnitude of clinical benefit analysis in the 2022 report was largely based upon ESMO-MCBS scores published by ESMO for specific solid tumour medicines. For some medicines, these published scores were not available. At the time of the 2024 report, published ESMO-MCBS:H scores for blood cancer medicine gaps were not available. For those medicines, the project team used the ESMO-MCBS:H to manually determine the clinical benefit scores.

This 2024 report re-evaluates the difference in blood cancer medicine availability between Aotearoa New Zealand and Australia and assesses the magnitude of clinical benefit for all identified medicine gaps using the recently released ESMO-MCBS:H. Re-evaluation of medicine availability and clinical benefit for solid tumour medicines was not within the scope of this complementary report.

What did the 2024 report involve?

This project had two key parts:

- Part 1: Determining the current difference in blood cancer medicine availability between Australia and Aotearoa New Zealand
- Part 2: Assessing the magnitude of clinical benefit for blood cancer medicine available in Australia but not in Aotearoa New Zealand.

[3] **www.esmo.org/guidelines/esmo-mcbs**

A primary project team was convened consisting of a pharmacist, a health economist, and haematologists. The general methodology was the same as that used for the 2022 report. Each part is explained in more detail below.

Part one: Determining differences in medicine availability

In this report, we compared the list of medicines for the treatment of blood cancers that are publicly funded in Australia with those that are publicly funded in Aotearoa New Zealand. From this comparison we were able to determine which blood cancer medicines are available in:

- both Australia and Aotearoa New Zealand
- Australia but not in Aotearoa New Zealand
- Aotearoa New Zealand but not in Australia.

A list of medicines publicly funded in Australia is available through the Pharmaceutical Benefits Scheme, which is a part of Australia's Department of Health and Aged Care. A list of medicines publicly funded in Aotearoa New Zealand is available through Pharmac's Pharmaceutical Schedule. We compared blood cancer medicine availability between the two lists as of 1 January 2024.

What was considered a 'gap'?

One cancer medicine may be used in the treatment of more than one cancer type or clinical circumstance. The specific clinical circumstance a medicine is used for is called the **indication**. An example of this is the blood cancer medicine pembrolizumab, which can be used for two indications: one being relapsed or refractory Hodgkin's lymphoma and the other being relapsed or refractory primary mediastinal B-cell lymphoma. Some cancer treatment consists of more than one cancer medicine being used at the same time (that is, used in combination) for a specific indication. The combination of treatments to treat a single indication is called a **regimen**.

In this report, gaps were defined in three ways:

1. **Individual medicine gaps (eg, azacitidine)**

   'Individual medicine gaps' refers to individual medicines that are funded in Australia but not in Aotearoa New Zealand irrespective of the specific clinical circumstance the medicine is used for (indication) or whether it needs to be used with another medicine at the same time (regimen).

2. **Medicine-indication pair gaps (eg, azacitidine for patients who are unfit for intensive chemotherapy)**

   'Medicine-indication pair gaps' refers to medicines that are funded in Australia but not in Aotearoa New Zealand, with reference to each specific and different indication for the medicine's use. One medicine might be funded for several indications.

3. **Regimen-indication pair gaps (eg, azacitidine with venetoclax for patients who are unfit for intensive chemotherapy)**

'Regimen-indication pair gaps' refers to the situation where more than one medicine will be required for a particular treatment and that all those medicines need to be funded to close that particular treatment gap.

Where a generic or biosimilar product is available in one country and the reference product (often called the originator, innovator, or brand-name product) was available in another, these were considered identical for the purpose of this analysis. Furthermore, if several medicines of the same class for the same indication were available in a jurisdiction, this was only considered once. For example, if medicine E and F are medicines from the same class with the same mechanism of action and were both funded in Australia for indication W, this would only be considered as a single gap. This is because only one of medicine E or F would need to be funded in Aotearoa New Zealand to close the identified gap.

Part two: Assessing the magnitude of clinical benefit of identified gaps

Once a gap was identified, the magnitude of clinical benefit of the individual medicine gaps was assessed using the ESMO-MCBS:H.

The ESMO-MCBS:H was developed to support the assessment of medicines in a more consistent, reliable and fair manner. It aims to highlight those medicines that have a significant positive impact on quality of life and/or survival and distinguish these medicines from those that offer marginal benefits or delay disease progression with no impact on a person's quality of life or survival.

To ensure the score determination process was robust and reliable, a two-step review and moderation process was carried out. In the first step, two members of the project team conducted independent evaluations of each medicine's magnitude of clinical benefit and determined a score. The two members then met to discuss their findings and moderate any differences. Once moderation was complete, the scoring forms were sent to two haematologists who independently reviewed the scores and how they were derived. Again, where difference in opinion occurred, the entire project team met to discuss and moderate the information and come to a resolution. Where there was some uncertainty, the project team favoured the highest score possible for the medicine.

The magnitude of clinical benefit score allocation relied on the assessment of outcomes from the specific clinical trial that led to the medicine being licensed. Some clinical benefit scores were amended for accuracy in the context of Aotearoa New Zealand. This was necessary because, at times, there was a difference between the medicine that was used as a comparator in the clinical trial to the medicine that would be used for that treatment purpose in Aotearoa New Zealand. Without allowing for this context, the score allocated might not have reflected the potential benefit offered by the medicine should it be made publicly available in Aotearoa New Zealand. Again, the project team erred on allocating the highest possible score for the medicine in this instance. Where several medicines with the same mode of action and used for the same indication were available in Australia, these were represented as a single medicine gap with the highest ESMO-MCBS:H score applied.

As an additional check, where possible the scores derived by the project team were checked against the ESMO-MCBS:H scores derived during the tool's evaluation and validation process[4].

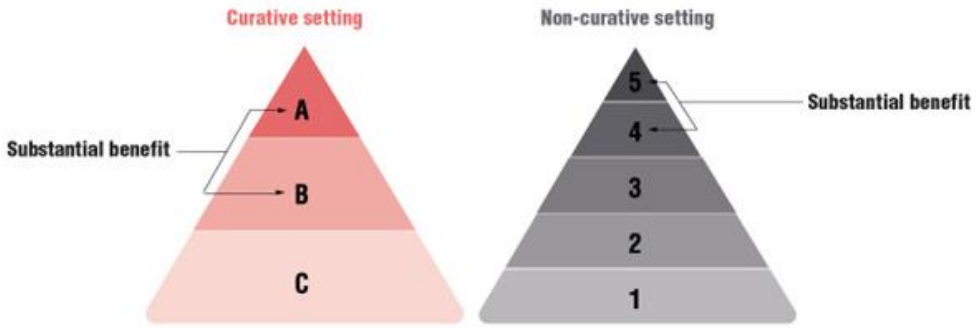
Using the ESMO-MCBS:H, the resulting scores can be:

- A, B or C – These scores indicate medicines that are used with curative intent. Medicines scored with an A or B indicate substantial clinical benefit would be achieved if they were available (Figure 1).
- 5, 4, 3, 2 or 1 – These scores indicate medicines that are used in a non-curative setting. That is, they improve quality of life or increase survival but will not cure the cancer. Medicines scored with a 5 or 4 indicate substantial clinical benefit would be achieved if they were available (Figure 1).
- No evaluable benefit (NEB) – These scores indicate that the trial evidence did not provide evidence of benefit.
- Non-scorable – This indicates the trial information was not able to be applied to the ESMO-MCBS:H.

Medicine-indication or regimen-indication pair gaps that were determined to have a substantial magnitude of clinical benefit were of primary interest in this analysis. For the purposes of this report, each medicine-indication pair gap was categorised as one of the following:

- Gap - substantial clinical benefit (ie, ESMO-MCBS score A, B, 4 or 5)
- Gap – not substantial clinical benefit (ie, ESMO-MCBS score C, 3, 2, 1)
- Gap – no evaluable benefit (NEB)
- Gap – not scorable

Figure 1: ESMO-MCBS:H possible scores

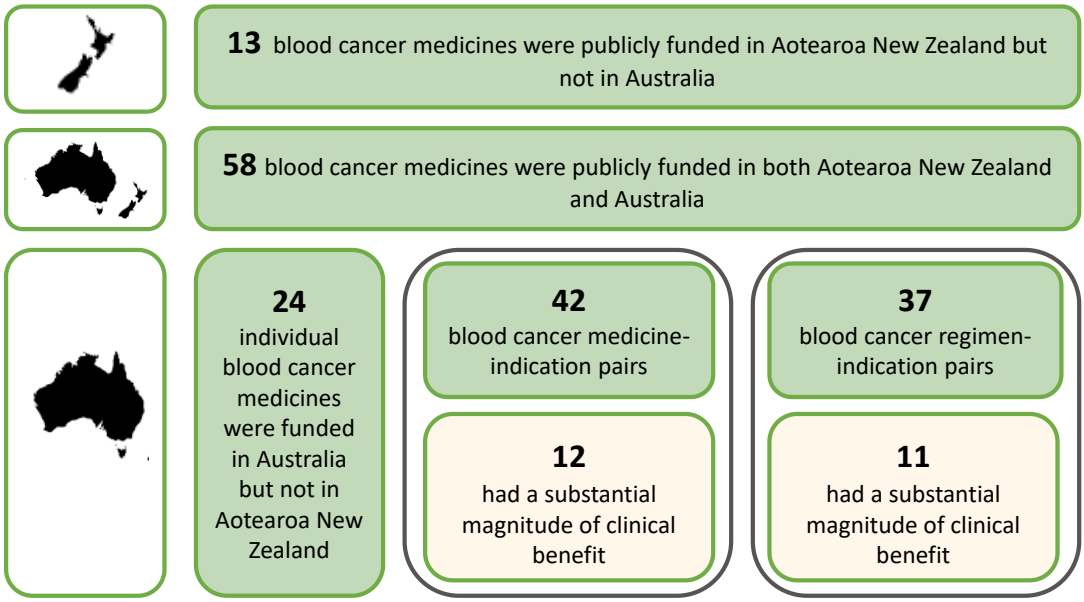


4 Kiesewetter B, Cherny N, Boissel, et al. 2020. EHA evaluation of the ESMO—Magnitude of Clinical Benefit Scale version 1.1 (ESMO-MCBS v1.1) for haematological malignancies. ESMO open 2020;(5). URL: https//doi:10.1136/esmoopen-2019-000611 (accessed 11 September 2024).

What did we find?

Figure 2 summarises the main findings of the blood cancer medicine availability analysis. The analysis showed that while there are many blood cancer medicines available in both Australia and Aotearoa New Zealand, there are more blood cancer medicines available in Australia than in Aotearoa New Zealand.

Figure 2: Summary of results in blood medicine availability between Australia and Aotearoa New Zealand on 1 January 2024



Of the 42 blood cancer medicine-indication pairs identified, 12 were determined to have a substantial magnitude of clinical benefit. This equated to 37 regimen-indication pairs. Two of the medicine-indication pairs were used in a curative setting and 10 were used in a non-curative setting. The blood cancer medicines with substantial magnitude of clinical benefit included four treatments for acute myeloid leukaemia, three for acute lymphoblastic leukaemia, two for multiple myeloma, and one for each of chronic myeloid leukaemia, non-Hodgkin's lymphoma and chronic lymphoblastic leukaemia respectively. Because some regimens involve treatment with more than one medicine at the same time, the 12 blood cancer medicine gaps represent 11 regimen-indication pair gaps identified as having a substantial magnitude of clinical benefit.

Appendix 1 summarises all medicines identified as being funded in Australia but not in Aotearoa New Zealand by tumour type and clinical benefit score.

Appendix 2 summarises the blood cancer medicines available in Australia but not in Aotearoa New Zealand that were determined to have a substantial magnitude of clinical benefit. The table presents information about each identified gap, including what it is used for, how it is administered and, if the identified gap is being considered by Pharmac for funding, what stage of assessment Pharmac is up to.

Appendix 3 summarises the blood cancer medicines available in Australia but not in Aotearoa New Zealand that were determined to have non-substantial magnitude of clinical benefit.

For more information on the identified gaps and other analysis results, refer to the 2024 full report.

What are the key strengths and limitations of this analysis?

This report goes beyond counting medicines, to consideration of medicine gaps according to their specific uses and the magnitude of clinical benefit of the identified medicines. One cancer medicine can be used to treat multiple different types and stages of cancer, so it is important to consider the medicine-indication and regimen-indication specific pairs. In addition, not all gaps are equal in terms of the health benefit Aotearoa New Zealand may achieve if a medicine was funded. Consideration of the identified gaps in terms of their magnitude of clinical benefit provides important context for understanding which gaps offer more potential for health benefit to Aotearoa New Zealand.

There are some important limitations to note about the methodology used in this report.

- While Australia has higher cancer medicine availability than Aotearoa New Zealand, it is important to remember that medicine availability in Australia is not reflective of an international 'gold standard'. There is variation in medicine availability between all countries, and there is not a 'right' or 'correct' level of medicine access. There may be blood cancer medicines not yet available in Australia that could provide significant health benefit to New Zealanders.
- The magnitude of clinical benefit scores in this report were derived from clinical trial evidence, which represents analysis at a group level. This means that there may be individuals who experience a different outcome when receiving a medicine. This analysis focuses on the outcomes of the treated group as an average.
- This report used the ESMO-MCBS:H to estimate clinical benefit. While this is an internationally validated tool, there are other methods available to determine clinical benefit that might have generated different findings.
- The ESMO-MCBS:H is very prescriptive in nature and is focused on assessment of evidence for increased survival or improved quality of life associated with the use of a medicine. It is also very prescriptive about what cannot be considered in the assessment of a medicine's magnitude of clinical benefit. This is both a strength and a limitation of the tool. It is a strength as it allows for consistent comparison of the magnitude of clinical benefit of medicines. However, some aspects of clinical trial quality are not considered by the ESMO-MCBS:H, and it does not allow for evidence of clinical benefit outside a clinical trial to be considered. Some scoring might be different when longer-term clinical data becomes available. The ESMO-MCBS:H also does not consider clinical trial information that was not part of the medicine becoming licensed. This might include valuable evidence that could alter clinical practice. Some medicines were unable to be scored because the information available in the trials did not meet the requirements of the ESMO-MCBS:H.
- The scores were derived in a robust process with as much quality control as possible. However, it is possible that the scores derived in this report will differ from those

published by ESMO when it was evaluating the ESMO-MCBS:H[5]. Some of these differences will be due to adaptations made to reflect the context in Aotearoa New Zealand.

- Decisions about the funding of medicines are complex, and Pharmac takes into consideration a wide range of factors to determine which medicines are included in the OFI list. The medicine gaps noted in this report count the numbers of medicines funded in Australia but not in Aotearoa New Zealand. They do not measure the health benefits across all New Zealanders that aren't achieved because those medicines are not available in Aotearoa New Zealand (that is, the number of people with each type of blood cancer affected, multiplied by how many healthy life years each person on average loses by not having the Australian-funded medicine).

Key things to consider when interpreting the findings of this report

Improving cancer outcomes requires a balance of actions across the continuum

While cancer medicines are a key part of cancer treatment, achieving fewer cancers, better survival and more equitable cancer outcomes for New Zealanders requires action across the entire cancer continuum (from prevention right through to palliative care). Decision making to determine how much money is invested in health, in cancer, and in cancer medicines specifically, is extremely complex. It is important that all action and investment in cancer treatment is balanced and coordinated to ensure that the limited resources in the wider cancer system achieve the best health outcomes possible for New Zealanders.

The impact of medicine availability on equity was out of scope

For medicines to provide benefit they need to be accessible as well as available. Some members of the population already experience inequity in accessing cancer services and treatment opportunities. This is likely to contribute to the known inequities in cancer health outcomes. This report does not include assessment of whether closing the gaps identified in this analysis would make a difference to equitable access to cancer treatment and to cancer outcomes.

[5] Kiesewetter B, Cherny N, Boissel, et al. 2020. EHA evaluation of the ESMO—Magnitude of Clinical Benefit Scale version 1.1 (ESMO-MCBS v1.1) for haematological malignancies. *ESMO open* 2020;(5). URL: https//doi:10.1136/esmoopen-2019-000611 (accessed 11 September 2024).

When medicines are funded, additional resources are also required

Providing New Zealanders with cancer treatment requires a coordinated team of health professionals and a series of health services and technologies. For funded medicines to provide their intended benefit, the health system needs a variety of trained health professionals (haematologists, oncologists, pharmacists, imaging specialists, nurses, and administrators), facilities (infusion sites, laboratory and imaging-processing sites, and pharmacies) and equipment. Funding new medicines that provide treatment to a group of people who previously would not have had a treatment, or to people who will now receive treatment for longer, means more of all these things are needed. It is important that appropriate infrastructure, including the health workforce, is considered when new medicine funding decisions are made to ensure new medicines can be administrated effectively, efficiently and equitably.

Decisions about medicine funding requires consideration of many factors in addition to clinical benefit

Decisions about medicines should not be made based on clinical benefit alone. As discussed earlier, funding decisions in Aotearoa New Zealand are made by Pharmac using the Factors for Consideration. This allows Pharmac to evaluate the benefit of a proposed new medicine and how this compares to other proposed new medicines, with reference to the medicines that are already available, the health needs of the patient population, the medicine suitability, and the cost implications of funding the new medicine.

HE KUPU WHAKAKAPI
CONCLUSION

Cancer medicines, provided alone or in combination with surgery and/or radiation therapy, are a critical part of cancer care. Medicines can give people the opportunity to be cured of cancer, increase their quality of life with cancer, or extend how long they live with cancer. People with cancer, their whānau, and their treatment team expect that the medicines they need will be available when they need them.

This report is a continuation of the 2022 report, which could not be completed for blood cancer medicines at that time. The same methodology was used to describe the difference in blood cancer medicine availability between Australia and Aotearoa New Zealand. The magnitude of clinical benefit of those gaps was also identified now that a validated tool has become available to do so.

Along with the findings of the 2022 report, this 2024 report provides important context and detail to better understand the reality of cancer medicine availability in Aotearoa New Zealand, as compared to Australia. The results of this analysis show that there are differences in the number of blood cancer medicines available in Australia compared to Aotearoa New Zealand and that some of those are likely to have a substantial clinical benefit, primarily in a non-curative setting.

The information presented in the 2022 and 2024 reports is designed to provide useful insights to people with cancer and their whānau, the health sector, Pharmac, the New Zealand Government, non-governmental organisations and the general public about the scale and magnitude of clinical benefit of medicine gaps in Aotearoa New Zealand.

While optimising the availability of cancer medicines and their role in improving health outcomes for people with cancer is particularly important, medicine availability cannot be considered in isolation. Coordinated and prioritised action across the entire cancer care continuum is required to ensure outcomes for New Zealanders with cancer improve to the greatest extent possible. The wider cancer health system, which works to prevent cancer and provide access to diagnosis and treatment across the cancer continuum, must be functioning well.

Te Aho o Te Kahu remains committed to working with all relevant stakeholders to strengthen services across the cancer care continuum and deliver on the goal of fewer cancers, better survival, and equity for all.

NGĀ ĀPITIHANGA
APPENDICES

Appendix 1: Summary of medicine availability gaps by type of cancer and ESMO-MCBS:H score

Blood cancer type	Total	Substantial clinical benefit			Not substantial clinical benefit				
		Curative	Non-curative						
		A*	5*	4*	3	2	1	NEB†	Not scorable
Leukaemia									
Acute lymphoblastic leukaemia	4	1	1	1	–	1	–	–	–
Acute myeloid leukaemia	6	1	2	1	–	–	–	–	2
Chronic lymphoblastic leukaemia	7	–	–	1	5	–	1	–	–
Chronic myeloid leukaemia	3	–	–	1	1	–	–	–	1
Lymphoma									
Hodgkin's lymphoma	1	–	–		1	–	–	–	–
Non-Hodgkin's lymphoma	5	–	–	1	1	–	–	2	1
Lymphoma B-cell	1	–	–	–	1	–	–	–	–
Lymphoma T-cell	4	–	–	–	1	1	2	–	–
Myeloma									
Multiple myeloma	7	–	–	2	3	2	–	–	–
Other									
Other‡	4	–	–	–	1	1	–	–	2
Total									
Total	42	2	3	7	14	5	3	2	6

Note: Gaps shown are for medicine-indication pairs. Total gaps are less if regimen-indication pairs were considered. No gaps had an ESMO-MCBS:H score of B or C.

* Indicates an ESMO-MCBS:H score of substantial clinical benefit.

† NEB = no evaluable benefit.

‡ 'Other' includes systemic light chain amyloidosis, myelodysplastic syndrome and chronic myelomonocytic leukaemia.

Appendix 2: Medicine-indication pairs funded in Australia but not in Aotearoa New Zealand with a substantial magnitude of clinical benefit

ESMO-MCBS:H score	Medicine name	Used in combination with	Cancer type	Indication per PBS	Formulation	Medicine funded in NZ for other indications	Current Pharmac status (Oct 2024)†	Noted as a gap in 2022
A Substantial clinical benefit – curative	Midostaurin	Anthracycline and cytarabine chemotherapy (both already funded in Aotearoa New Zealand)	Acute myeloid leukaemia (AML)	Newly diagnosed patients with an internal tandem duplication (ITD) or tyrosine kinase domain (TKD) FMS-like tyrosine kinase 3 (FLT3) mutation	Oral capsule	No	Funded from 1 July 2024	Yes
A Substantial clinical benefit – curative	Blinatumomab	Monotherapy	Precursor B-cell acute lymphoblastic leukaemia (pre-B-cell ALL)	Complete haematological remission with measurable residual disease (MRD)	Continuous intravenous infusion	No	Seeking clinical advice	Yes
5 Substantial clinical benefit – not curative	Azacitidine*	Venetoclax*	Acute myeloid leukaemia (AML)	Patients who are unfit for intensive chemotherapy	Subcutaneous injection or intravenous infusion	Yes	OFI list	No
5 Substantial clinical benefit – not curative	Blinatumomab	Monotherapy	Precursor B-cell acute lymphoblastic leukaemia (pre-B-cell ALL)	Patients with relapsed or refractory disease	Continuous intravenous infusion	No	No application	Yes
5 Substantial clinical benefit – not curative	Venetoclax*	Azacitidine*	Acute myeloid leukaemia (AML)	Patients who are unfit for intensive chemotherapy	Oral tablet	Yes	OFI list	No

ESMO-MCBS:H score	Medicine name	Used in combination with	Cancer type	Indication per PBS	Formulation	Medicine funded in NZ for other indications	Current Pharmac status (Oct 2024)[†]	Noted as a gap in 2022
4 Substantial clinical benefit – not curative	Acalabrutinib, ibrutinib or zanubrutinib[‡]	Monotherapy	Mantle cell lymphoma (MCL)	Relapsed or refractory to at least one prior therapy	Oral capsule/ tablet	Yes	Acalabrutinib – no application Ibrutinib – OFI list Zanubrutinib – OFI list	Yes
4 Substantial clinical benefit – not curative	Asciminib	Monotherapy	Chronic myeloid leukaemia (CML)	Philadelphia chromosome positive or with transcript BCR-ABL1 tyrosine kinase mutation positive without T315I mutation in chronic phase previously treated with two or more tyrosine kinase inhibitors	Oral tablet	No	Seeking clinical advice	No
4 Substantial clinical benefit – not curative	Gilteritinib	Monotherapy	Acute myeloid leukaemia (AML)	Relapsed or refractory with FLT3 ITD or TKD mutation	Oral tablet	No	No application	No
4 Substantial clinical benefit – not curative	Idelalisib	Rituximab for 8 doses followed by monotherapy (already funded in Aotearoa New Zealand)	Chronic lymphocytic leukaemia (CLL) or small lymphocytic lymphoma (SLL)	Relapsed or refractory to at least one prior therapy, CD20-positive	Oral tablet	No	No application	Yes
4 Substantial clinical benefit – not curative	Inotuzumab ozogamicin	Monotherapy	Precursor B-cell acute lymphoblastic leukaemia (pre-B-cell ALL)	Relapsed or refractory B-precursor cell, CD22-positive	Intravenous infusion	No	OFI list	Yes

ESMO-MCBS:H score	Medicine name	Used in combination with	Cancer type	Indication per PBS	Formulation	Medicine funded in NZ for other indications	Current Pharmac status (Oct 2024)†	Noted as a gap in 2022
4 Substantial clinical benefit – not curative	Lenalidomide	Dexamethasone with or without bortezomib	Multiple myeloma (MM)	Newly diagnosed	Oral capsule/ tablet	Yes	Funded from 1 August 2024	Yes
4 Substantial clinical benefit – not curative	Pomalidomide	Dexamethasone (already funded in Aotearoa New Zealand)	Multiple myeloma (MM)	Relapsed or refractory third-line treatment	Oral capsule	No	Funded from 1 August 2024	Yes

Note: a score of A or B indicates curative, a score of 5 or 4 indicates substantial clinical benefit but not curative.

* Represents a regimen-indication pair – where more than one medicine is taken as part of the same treatment, and both would need to be funded to close the gap.

† Status as of May 2024. The status of medicine applications at Pharmac is constantly being progressed and updated. Please refer to Pharmac's Application Tracker (connect.pharmac.govt.nz/apptracker) for up-to-date information on a medicine application.

‡ Medicines that are part of the Bruton's tyrosine kinase (BTK) inhibitors medicine class – only one medicine from the medicine classes would need to be funded to close the identified gap. The ESMO-MCBS:H score reflects the highest score of the medicines scored in the class. Differences in ESMO-MCBS:H score are likely due to differences in trial design, follow-up periods and available data.

Appendix 3: Medicine-indication pairs funded in Australia but not in Aotearoa New Zealand that were determined to not have a substantial magnitude of clinical benefit

ESMO-MCBS:H score	Medicine name	Used in combination with	Cancer type	Indication per PBS	Formulation	Medicine funded in NZ for other indications	Current Pharmac status (October 2024)*	Noted as a gap in 2022
3	Acalabrutinib, ibrutinib or zanubrutinib†	Monotherapy	Chronic lymphocytic leukaemia (CLL) or small lymphocytic lymphoma (SLL)	Relapsed or refractory, second line, prior therapy, TP53 wildtype.	Oral capsule/ tablet	No	Acalabrutinib – Under-assessment Ibrutinib – Under assessment Zanubrutinib – Under assessment	Yes
3	Acalabrutinib‡	Obinutuzumab‡	Chronic lymphocytic leukaemia (CLL) or small lymphocytic lymphoma (SLL)	Untreated patients or those who have developed an intolerance resulting in withdrawal from another first-line agent	Oral capsule/ tablet	No	No application	No
3	Carfilzomib	Dexamethasone with or without lenalidomide	Multiple myeloma (MM)	Progressive disease after at least one prior therapy (once or twice weekly)	Intravenous infusion	No	OFI list	Yes
3	Daratumumab§	Bortezomib and dexamethasone	Multiple myeloma (MM)	Progressive disease after only one prior therapy	Intravenous infusion/ subcutaneous injection	No	OFI list	Yes
3	Elotuzumab‡	Dexamethasone and lenalidomide¶	Multiple myeloma (MM)	Progressive disease after at least one prior therapy	Intravenous infusion	No	No application	No

3	Idelalisib	Monotherapy	Follicular B-cell non-Hodgkin's lymphoma	Refractory to rituximab and an alkylating agent within 6 months after completion of the treatment	Oral tablet	No	No application	Yes
3	Lenalidomide	Monotherapy	Myelodysplastic syndrome	Low risk or intermediate-1 with del(5q), and red blood cell transfusion dependent	Oral capsule/ tablet	Yes	Funded from 1 August 2024	Yes
3	Obinutuzumab‡	Acalabrutinib‡	Chronic lymphocytic leukaemia (CLL) or small lymphocytic lymphoma (SLL)	Untreated patients or those who have developed an intolerance resulting in withdrawal from another first-line agent	Intravenous infusion	Yes	No application	No
3	Obinutuzumab‡	Venetoclax‡	Chronic lymphocytic leukaemia (CLL) or small lymphocytic lymphoma (SLL)	Previously untreated disease	Intravenous infusion	Yes	Under assessment	Yes
3	Pembrolizumab	Monotherapy	Hodgkin's lymphoma	Relapsed or refractory Hodgkin's lymphoma after autologous haematopoietic stem cell transplant, and if transplant ineligible, has relapsed after 2 prior treatments	Intravenous infusion	Yes	Funded from 1 October 2024	Yes
3	Pembrolizumab	Monotherapy	Primary mediastinal B-cell lymphoma	Relapsed or refractory after autologous stem cell transplant, or after 2 prior treatments and if transplant ineligible has relapsed after 1 prior treatment. Patient must have been treated with rituximab	Intravenous infusion	Yes	No application	Yes

3	Ponatinib	Monotherapy	Chronic myeloid leukaemia (CML)	At least two prior tyrosine kinase inhibitors have failed or have not been tolerated with a severity necessitating permanent treatment withdrawal	Oral tablet	No	No application	Yes
3	Pralatrexate	Not specified	Peripheral T-cell lymphoma	Relapsed or chemotherapy-refractory after appropriate prior first-line curative intent chemotherapy	Intravenous bolus	No	No application	Yes
3	Venetoclax‡	Obinutuzumab‡	Chronic lymphocytic leukaemia (CLL) or small lymphocytic lymphoma (SLL)	Previously untreated disease	Oral tablet	Yes	Under assessment	Yes
2	Brentuximab vedotin	Cyclophosphamide, doxorubicin and prednisone	Peripheral T-cell lymphoma, non-cutaneous	First-line treatment with curative intent, CD30-positive	Intravenous infusion	Yes	Under assessment for anaplastic large cell lymphoma only	Yes
2	Daratumumab	Cyclophosphamide, bortezomib and dexamethasone	Systemic light chain (AL) amyloidosis	Newly diagnosed	Intravenous infusion/ subcutaneous injection	No	OFI list	No
2	Pomalidomide	Dexamethasone and bortezomib	Multiple myeloma (MM)	Progressive disease after at least one prior therapy (that is either lenalidomide monotherapy or contains lenalidomide) and patient has undergone or is ineligible for an autologous haematopoietic stem cell transplant	Oral capsule	No	Funded from 1 August 2024	No

2	Ponatinib	Monotherapy	Acute lymphoblastic leukaemia (ALL)	Second line treatment for patients with T315I, Philadelphia chromosome positive or BCR-ABL mutation	Oral tablet	No	No application	Yes
2	Selinexor	Triplet with dexamethasone and bortezomib. Doublet with dexamethasone	Multiple myeloma (MM)	Progressive disease after at least one prior therapy	Oral tablet	No	No application	No
1	Brentuximab vedotin	Monotherapy	T-cell lymphoma, cutaneous	Relapsed or refractory to prior treatment, CD30-positive	Intravenous infusion	Yes	No application	Yes
1	Vorinostat	Monotherapy	T-cell lymphoma, cutaneous	Relapsed or chemotherapy-refractory and ineligible for autologous haematopoietic stem cell transplant	Oral capsule	No	No application	Yes
1	Acalabrutinib or zanubrutinib†	Monotherapy	Chronic lymphocytic leukaemia (CLL) or small lymphocytic lymphoma (SLL)	Previously untreated or intolerant to another first-line drug treatment	Oral capsule	No	Acalabrutinib – no application Zanubrutinib – no application for whole group. Application for 17p deletion or TP53 mutation disease, first line specifically – OFI list	No
NEB	Bendamustine‡	Obinutuzumab‡ or rituximab	Follicular lymphoma stage II bulky or stage III/IV	Induction, previously untreated, CD20-positive	Intravenous infusion	Yes	No application	Yes

NEB	Obinutuzumab	Bendamustine‡	Follicular lymphoma stage II bulky or stage III/IV	Induction, previously untreated, CD20-positive	Intravenous infusion	Yes	Application declined	Yes
Not scorable	Asciminib	Monotherapy	Chronic myeloid leukaemia (CML)	Philadelphia chromosome positive or with transcript BCR-ABL1 tyrosine kinase chronic myeloid leukaemia in chronic phase with the T315I mutation	Oral tablet	No	No application	No
Not scorable	Azacitidine	Monotherapy	Acute myeloid leukaemia (AML)	Intermediate or poor risk at diagnosis. Treatment follows intensive induction chemotherapy, with complete remission, in patients who have not undergone or are not proceeding to allogeneic haematopoietic stem cell transplant	Oral tablet	Yes	No application	No
Not scorable	Decitabine with cedazuridine	Not specified	Acute myeloid leukaemia (AML)	With 20%–30% marrow blasts and multi-lineage dysplasia	Oral tablet	No	No application	No
Not scorable	Decitabine with cedazuridine	Not specified	Chronic myelomonocytic leukaemia (CMML)	With 10%–29% marrow blasts without myeloproliferative disorder	Oral tablet	No	No application	No
Not scorable	Decitabine with cedazuridine	Not specified	Myelodysplastic syndrome	Intermediate-2 or high risk 20% marrow blasts	Oral tablet	No	No application	No
Not scorable	Zanubrutinib	Monotherapy	Waldenstrom macroglobulinaemia	Relapsed or be refractory to at least one prior chemo-immunotherapy or be unsuitable for treatment with chemo-immunotherapy	Oral capsule	No	Under assessment	No

Definitions: 'Monotherapy' means the medicines are taken by themselves; 'NEB' means no evaluable benefit; 'Not scoreable' primarily indicates clinical trials with outcomes that cannot be assessed using the ESMO-MCBS:H.

* Status as of May 2024. The status of medicine applications at Pharmac is constantly being progressed and updated. Please refer to Pharmac's Application Tracker for up-to-date information on a medicine application (**connect.pharmac.govt.nz/apptracker/s**).

† Medicines that are part of the Bruton's tyrosine kinase (BTK) inhibitors medicine class – only one medicine from the medicine classes would need to be funded to close the identified gap. The ESMO-MCBS:H score reflects the highest score of the medicines scored in the class. Differences in ESMO-MCBS:H score are likely due to differences in trial design, follow-up periods and available data.

‡ Represents a regimen-indication pair – where more than one medicine is taken as part of the same treatment, and both would need to be funded to close the gap.

§ The ESMO-MCBS:H score for daratumumab reflects the intention to treat the population of the CASTOR trial (Hungria et al 2021), which included patients using daratumumab second line treatment or later. The analysis that examined the benefit of taking daratumumab as a second-line treatment (ie, the population funded in Australia) was unable to be scored by the ESMO-MCBS:H as it was a post-hoc analysis (ie, an analysis that was not planned to be conducted at the beginning of the trial).

¶ Lenalidomide is funded in Aotearoa New Zealand with a Special Authority restricting use to people with relapsed or refractory multiple myeloma with progressive disease as a third-line treatment, with second-line use permitted in the presence of dosing limiting neuropathy to first-line agents. Clinical advice received indicated that only a small number of patients will not be covered by the current access criteria.

Appendix 4: Key sources of information

Information type	Source(s)	Link(s) (if applicable)
Medicines funded in Aotearoa New Zealand	Pharmac's Pharmaceutical Schedule	schedule.pharmac.govt.nz/ScheduleOnline.php schedule.pharmac.govt.nz/HMLOnline.php
Medicines funded in Australia	Pharmaceutical Benefits Scheme Schedule	www.pbs.gov.au/browse/body-system
Indications for medicines funded without restriction	Therapeutic Goods Administration product information	www.ebs.tga.gov.au
	eviQ	www.eviq.org.au
	Medsafe Data Sheets	www.medsafe.govt.nz/Medicines/infoSearch.asp
ESMO-MCBS:H scores	ESMO	www.esmo.org/guidelines/esmo-mcbs/esmo-mcbs-for-haematological-malignancies/esmo-mcbs-h-evaluation-forms
Pharmac status	Pharmac Application Tracker	connect.pharmac.govt.nz/apptracker/s
Clinical relevance of gaps	Clinical advice	
	ACT-NOW SACT regimen library	nzf.org.nz/regimens
	eviQ	www.eviq.org.au
Patient and health sector considerations	ACT-NOW SACT regimen library	nzf.org.nz/regimens
	eviQ	www.eviq.org.au
	Medsafe Data Sheets	www.medsafe.govt.nz/Medicines/infoSearch.asp