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Specialist Advisory Committees
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Record of the Cancer Treatments Advisory Committee Meeting held on 30 April 2026

Cancer Treatments Advisory Committee records are published in accordance with the Terms of Reference for the Specialist Advisory Committees 2021.

Note that this document is not necessarily a complete record of the Cancer Treatments Advisory Committee meeting; only the relevant portions of the meeting record relating to Cancer Treatments Advisory Committee discussions about an application or Pharmac staff proposal that contain a recommendation are generally published.

The Cancer Treatments Advisory Committee may:

(a) recommend that a pharmaceutical be listed by Pharmac on the Pharmaceutical Schedule and the priority it gives to such a listing;

(b) defer a final recommendation, and give reasons for the deferral (such as the supply of further information) and what is required before further review; or

(c) recommend that Pharmac decline to list a pharmaceutical on the Pharmaceutical Schedule.

Pharmac Advisory Committees make recommendations, including priority, within their therapeutic groups of interest.

The record of this Advisory Committee meeting will be reviewed by PTAC at an upcoming meeting.

Specialist Advisory Committees and PTAC may differ in the advice they provide to Pharmac, including recommendations' priority, due to the committees' different, if complementary, roles, expertise, experience, and perspectives.

Pharmac is not bound to follow the recommendations made below. Applications are prioritised by Pharmac against other funding options and progressed accordingly. The relative priority of any one funding choice is dependent on a number of factors, including (but not limited to) the recommendation of PTAC and/or Specialist Advisory Committees, the mix of other applications being assessed, the amount of funding available, the success of commercial negotiations and/or the availability of clinical data.

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1. Attendance

Present
Stephen Munn
Alice Minhinnick
Allanah Kilfoyle
Chris Frampton
Matthew Strother
Lochie Teague
Scott Babington
Vidya Mathavan

Observers
Anita Fitzgerald
James Le Fevre

Apologies
Michelle Wilson
Richard Isaacs

2. Summary of recommendations

	Pharmaceutical and Indication	Recommendation
8.3	Alectinib widened access for the adjuvant treatment of resected, anaplastic lymphoma kinase-positive (ALK-positive) non-small cell lung cancer	Medium Priority
9.2	Durvalumab for the treatment of limited-stage, small cell lung cancer	High priority
10.4	Inavolisib (in combination with palbociclib and endocrine therapy) for the treatment of HR-positive, HER2-negative locally advanced or metastatic breast cancer with a *PIK3CA*-mutation, following recurrence on/after adjuvant therapy	Medium priority
11.3	Ibrutinib widened access for the first line treatment of mantle cell lymphoma (with funding of rituximab maintenance extended to three years for this group)	High priority
12.3	Blinatumomab for the treatment of MRD-negative B-cell lineage acute lymphoblastic leukaemia	High priority

These recommendations were made within the context of treatments of malignancy.

3. The role of Specialist Advisory Committees and records of meetings

3.1. This meeting record of the Cancer Treatments Advisory Committee is published in accordance with the Terms of Reference for the Pharmacology and Therapeutics Advisory Committee (PTAC) 2021 and Specialist Advisory Committees 2021.Terms of

Reference describe, *inter alia*, the establishment, activities, considerations, advice, and the publication of such advice of Specialist Advisory Committees and PTAC.

3.2. Conflicts of Interest are described and managed in accordance with section 6.4 of the SAC Terms of Reference.

3.3. The Cancer Treatments Advisory Committee is a Specialist Advisory Committee of Pharmac. The Cancer Treatments Advisory Committee and PTAC and other Specialist Advisory Committees have complementary roles, expertise, experience, and perspectives. The Cancer Treatments Advisory Committee and other Specialist Advisory Committees may therefore, at times, make recommendations for treatments for cancer that differ from PTAC's, including the priority assigned to recommendations, when considering the same evidence. Likewise, PTAC may, at times, make recommendations for treatments for cancer that differ from the Cancer Treatments Advisory Committee's, or Specialist Advisory Committees may make recommendations that differ from other Specialist Advisory Committees'.

Pharmac considers the recommendations provided by both the Cancer Treatments Advisory Committee and PTAC and any other relevant Specialist Advisory Committees when assessing applications for treatments for cancer.

4. Welcome and introduction

4.1. The Chair welcomed the Committee with a karakia followed by whakawhanaungatanga.

5. Record of meetings held September & October 2025

5.1. The Committee reviewed the record of the meetings held on 12 September and 9 October 2025 and agreed that the records be accepted.

6. Pharmac Update

6.1. The Committee noted the Pharmac Update.

6.2. The Committee acknowledged the recent staff updates and noted the ongoing recruitment for CTAC.

6.3. The Committee noted key updates regarding:

6.3.1. the organisational Reset Programme

6.3.2. the Exceptional Circumstances Framework review and consultation

6.4. The Committee noted the agenda item for Rule 8.1b which will expand on previous discussion.

6.5. The Committee noted and acknowledged the summary of applications waiting for clinical advice and the updated workplan for the Committee across 2026 meetings.

6.6. The Committee noted that two Health New Zealand observers will attend item 11 on the agenda to help inform how Health New Zealand and Pharmac will assess companion diagnostic tests, a medical device category that sits with Health New Zealand and links closely with the relevant medicine.

7. Matters arising - Alectinib ECOG status change for advanced non-small cell lung cancer (ALK-positive)

Recommendations

7.1. The Committee supported that funding of alectinib for treatment of advanced ALK+ NSCLC be widened to permit use in people with Eastern Cooperative Oncology Group (ECOG) Performance Score (PS) 3 where poor PS is thought to be driven by the underlying disease and treatment is expected to have a significant impact on PS. The Committee noted the potential changes to the criteria be refined and considered further at a future CTAC meeting.

Discussion

Background

7.2. The Committee noted alectinib for the treatment of advanced anaplastic lymphoma kinase positive (ALK+) non-small cell lung cancer (NSCLC) was funded in October 2019 for individuals with an Eastern Cooperative Oncology Group (ECOG) Performance Score (PS) 0-2.

7.3. The Committee noted the Lung Oncology Special Interest Group (LOSIG) provided feedback to Pharmac requesting that the current funding criteria for alectinib be expanded to include people with an ECOG PS of 3. The correspondence from the LOSIG included supporting evidence for the use of alectinib in poorer PS populations from the literature.

Discussion of the evidence provided

7.4. The Committee considered that the quality of studies identified by the LOSIG was variable, and that the evidence presented was largely subjective and not entirely representative of the overall ECOG 3 population.

7.5. The Committee considered there was a lack of robust data from comparable health systems regarding the proportion of ALK-mutated NSCLC populations with each ECOG PS score at presentation.

7.6. The Committee noted evidence reported by Singh A et al. ecancermedicalscience. 2022 ;16:1407 that approximately 30% of people had an ECOG of 3-4. The Committee considered that this population was located in an Indian healthcare setting where treatment access constraints may limit generalisability.

7.7. The Committee noted evidence by Masuda N et al. Cancer Sci. 2019;110:1401–07 which reported approximately 5% of the population had an ECOG of 3. The Committee considered this was consistent with other evidence from Ueno N, Banno S. Jpn J Clin Oncol. 2019 Jul;49:676–686 in the Japanese health system, which reported approximately 13% ECOG 2, 6% ECOG 3, and 2.9% ECOG 4. The Committee considered the evidence was broadly generalisable to the New Zealand context.

7.8. The Committee noted US-based real-world evidence derived from the Flatiron datasets, including Bauman JR, Liu G. Lung Cancer. 2024 Sep;195:107919 and Zhang Q, Lin JJ. JTO Clin Res Rep. 2023 Apr;4:100483. The Committee noted that these studies reported approximately 11% of people had ECOG ≥2 and approximately 3.5% had ECOG 3 respectively. The Committee considered these proportions were generalisable to the New Zealand context.

7.9. The Committee considered that, across comparable healthcare systems, the proportion of people with ECOG ≥2 in the first-line treatment setting is likely to range between approximately 3% and 11%.

7.10. The Committee considered that the proportion of people with an ECOG of 3 remains uncertain but is likely in the range of approximately 3% to 5%.

7.11. The Committee considered that the uptake of alectinib in this population could be high, as most people in this population currently receive best supportive care (BSC). This was supported by the correspondence from the LOSIG.

7.12. The Committee considered that, at present, a very small number of people receive chemotherapy in the ALK+ NSCLC ECOG 3 population. The Committee considered that while some individuals with poor ECOG related to brain metastases receive radiation, the majority of these people would receive BSC. The Committee considered it is likely that people with ALK+ NSCLC ECOG 3 are often assessed as ECOG 2 where the clinician thinks the person would benefit from alectinib treatment.

7.13. The Committee considered that alectinib would be less well tolerated than BSC in the ECOG 3 population compared to individuals with better ECOG PS.

7.14. The Committee noted that the group with an ECOG PS of 3 is a small population lacking strong data in either the trial setting or real-world evidence. The Committee further considered that ECOG scoring in real world practice is highly subjective, which introduces uncertainty into available real-world evidence.

7.15. The Committee considered that a decline in PS may be driven by underlying disease, particularly central nervous system (CNS) disease or brain metastases, and ALK mutation status, and that effective treatment could improve ECOG score where functional impairment is disease-driven. The Committee considered that when people in this situation experienced a treatment response, continued treatment was likely to be similarly effective for them as the group with current access to alectinib.

7.16. The Committee noted the Kyushu 1401 study (Iwama E et al. The Oncologist. 2020;25:306–e618) included individuals who were ECOG 3–4 treated with alectinib and considered that this evidence supported increasing the ECOG threshold to 3. However, the Committee also considered that data from this study were limited by the small sample size and was insufficient to draw reliable conclusions.

7.17. Members considered that an option may be to remove the ECOG restriction from the Special Authority criteria.

7.18. The Committee considered that while responses to treatment would be expected rapidly (approximately two weeks) in some people, such improvements would not be common. This was especially the case where a higher ECOG PS was not driven by underlying disease. Hence, the overall benefit at a population level, of widening access to people with any ECOG score was considered to be notably lower than in the currently funded population. Therefore, the Committee considered removing ECOG criteria entirely would be likely to reduce the average population level health benefit of alectinib.

7.19. Members considered the Special Authority criteria would need to balance the utility of flexible ECOG criteria in the context of highly responsive tumours or highly effective therapy, with the need for ECOG as a valid decision-making criterion. Members considered that clinicians would be able to clearly identify which people would gain the most benefit from this amendment, as only a small subgroup of people with a higher ECOG score would be expected to significantly benefit from treatment - due to their ECOG score being raised by their underlying disease.

7.20. Members considered the following potential Special Authority criteria wording: ECOG 3 where PS is due to underlying disease, and the person has a high probability of response, with review at six weeks.

7.21. The Committee considered it was likely there would be a high rate of treatment discontinuation, however the exact number was uncertain.

7.22. The Committee considered that, among solid tumours in general, poor performance by ECOG PS occurs in a relatively high proportion of cases (~30%). It was considered that treatment benefits would commonly be lower in people with poorer PS, and that only a very small proportion may respond well to treatment. The Committee considered the group of people with higher ECOG scores (≥3) with NSCLC specifically, who would be expected to respond well to treatment due to their PS being worsened by their underlying disease, to be relatively well-defined. For these people, the Committee considered that the benefit of treatment would likely be the same as the wider population. It was further considered likely that such a well-defined group may not necessarily exist in the context of other solid tumours, so targeting treatment to people with higher ECOG scores who may benefit significantly from treatment would not always be possible.

Funding criteria

7.23. Members considered the possibility of limiting treatment to a short initial duration to manage risk, with treatment continuation restricted to people whose disease responded well.

7.24. The Committee considered that alectinib may have greater health benefit in individuals with a higher ECOG due to alectinib's CNS penetration, and that this may not be generalisable across all tumour types, beyond NSCLC.

7.25. Members considered that risk of continued treatment without benefit for non-responders could be mitigated through response-based renewal criteria.

7.26. The Committee considered that imaging at approximately six weeks would occur as part of standard practice, possibly limiting the need to reflect this explicitly in any proposed criteria.

7.27. Members considered multiple potential approaches, including removal of ECOG restrictions; introduction of renewal criteria with stopping rules; or limiting use to cases where ECOG status is considered driven by underlying disease and expected to improve with treatment.

7.28. The Committee was supportive of amending the Special Authority criteria to allow for treatment of people with an ECOG PS of 3 where poor PS is driven by the underlying disease and has a high chance of benefiting the person. The Committee requested that Pharmac develop and refine the proposed criteria wording based on this recommendation for future Committee review.

8. Alectinib for non-small cell lung cancer (NSCLC)

Application

8.1. The Committee reviewed an application for alectinib in the adjuvant treatment of ALK-positive resected non-small cell lung cancer.

8.2. The Committee took into account, where applicable, Pharmac's relevant decision-making framework when considering this agenda item.

Recommendation

8.3. The Committee **recommended** that access to alectinib be widened with a **medium priority** for the adjuvant treatment of resected, anaplastic lymphoma kinase-positive (ALK-positive) non-small cell lung cancer, within the context treatments for malignancy, subject to the following Special Authority criteria:

Alectinib (non-small cell lung cancer, ALK-positive, resected)

Initial application – Applications from any relevant practitioner. Approvals valid for 6 months meeting the following criteria:
All of the following:

1. Individual must have had complete resection of non-small cell lung cancer with negative margins (tumour(s) ≥4 cm or node positive); and
2. There is documentation confirming that the patient has an ALK tyrosine kinase gene rearrangement using an appropriate ALK test

Renewal – Applications from any relevant practitioner. Approvals valid for 6 months meeting the following criterion

1. No evidence of progressive disease.

Note: Treatment may be given for a maximum of two years.

8.4. In making their recommendation, the Committee considered:

8.4.1. the high health need for people with ALK-positive non-small cell lung cancer, including the higher incidence and poorer outcomes seen in Māori and Pacific peoples

8.4.2. treatment with alectinib is associated with a significant and clinically meaningful improvement in disease-free survival, with a moderate likelihood that this will translate into an overall survival benefit

8.4.3. alectinib is an oral treatment, which is convenient for patients, but requires up to two years of therapy, compared with around three months of adjuvant chemotherapy in current practice.

8.5. The Committee suggested it relook at the proposal for osimertinib for epidermal growth factor receptor **(**EGFR)-positive NSCLC at a future meeting, which had a recommendation previously deferred.

Discussion

Māori impact

8.6. The Committee discussed the impact of funding alectinib for the treatment of resected ALK-positive NSCLC on Māori health areas of focus | Hauora Arotahi and Māori health outcomes. Members noted that the treatment of lung cancer is one of the five Māori health areas of focus | Hauora Arotahi.

Populations with high health needs

8.7. The Committee discussed the health need(s) of individuals with resected ALK-positive NSCLC among Māori, Pacific peoples, disabled peoples including tāngata whaikaha Māori, and other populations identified by the Government Policy Statement on Health 2024-2027 to have high health needs. The Committee discussed the impact of widening access to alectinib and considered:

8.7.1. Māori and Pacific peoples are disproportionately affected by lung cancer, with age-standardised incidence rates of 73.2 and 43 per 100,000 population, respectively, compared with 22.9 per 100,000 among European/other populations between 2019–2023 (Te Whatu Ora | Health New Zealand. Cancer Data Web Tool).

8.7.2. Between 2019-2022, Māori and Pacific peoples reported a lower curative resection rate (16.8% and 18.4%, respectively) compared to Europeans (19.1%) (Te Aho O Te Kahu | Cancer Control Agency. 2025. Lung Cancer Quality Improvement Monitoring Report Update).

8.7.3. The increased age-standardised mortality rate for Māori (51.9 per 100,000 people) and Pacific peoples (29.8 per 100,000 people) compared to European/other ethnicities (16.3 per 100,000 people) (Cancer Data Web Tool).

8.7.4. Socioeconomic status is associated with increased lung cancer incidence and mortality (Cancer Control Agency | Te Aho O Te Kahu. 2020. The State of Cancer In New Zealand).

8.7.5. The findings of McKeage et al. Intern Med J. 2020;50:716-25, which reported the prevalence of positive ALK-gene rearrangement in NSCLC to be higher for Māori (6.9%) and Pacific peoples (10.8%) compared to people of European ethnicity (4.4%).

Background

8.8. The Committee noted alectinib is already funded as the first line treatment for advanced ALK-positive NSCLC. The Committee noted the current application requested widened access to alectinib to include the adjuvant treatment of resected NSCLC.

8.9. The Committee considered there to be increasing attention to adjuvant, neoadjuvant and perioperative treatments aimed at improving the modest benefits associated with adjuvant chemotherapy. Members noted having previously reviewed other regimens in this setting, including:

8.9.1. neoadjuvant nivolumab (with chemotherapy) for resectable NSCLC (2025-11 CTAC record)

8.9.2. adjuvant atezolizumab for resected PD-L1-postive NSCLC (2025-09 CTAC record)

8.9.3. adjuvant osimertinib for resected EGFR-positive NSCLC (2023-07 CTAC record).

8.10. The Committee considered that testing for the ALK gene rearrangement was standard practice in New Zealand, mostly by immunohistochemistry (IHC) or by fluorescence in situ hybridisation (FISH).

Health need

8.11. The Committee noted that the health need associated with resectable NSCLC was reviewed by CTAC in November 2025. The Committee considered there was a high health need in this setting, reiterating that:

8.11.1. lung cancer is the leading cause of cancer-related mortality in New Zealand (Te Aho o Te Kahu. 2025)

8.11.2. approximately 30-50% of people present with resectable NSCLC (Kris et al. Transl Lung Cancer Res. 2023;12:824-36). The Committee considered that not all people with resectable NSCLC are suitable candidates for surgery

8.11.3. in New Zealand, 18.9% of people with NSCLC received a surgical resection between 2019-2022 (Te Aho o Te Kahu. 2025)

8.11.4. the Committee noted compared to surgery alone, adding adjuvant chemotherapy is associated with a modest 5.4% increase in 5-year overall survival (OS) for people presenting with all stages of NSCLC (Pignon et al. J Clin Oncol. 2008;26:3552-3559)

8.11.5. the Committee noted 25-55% of people diagnosed with stage I-II NSCLC will die of recurrent disease (Rami-Porta et al. J Thorac Oncol. 2015;10:990-1003.

8.12. The Committee noted that approximately 4–5% of NSCLC are ALK-positive. ALK-positive disease is more commonly observed in non-smokers and younger patients, often presents at a more advanced stage, and is associated with a high risk of developing brain metastases (Griesinger et al. Oncotarget, 2018;9:35181-84).

8.13. The Committee considered NSCLC to be a highly morbid disease, characterised by respiratory symptoms and symptoms from metastatic disease sites for example bone pain, and associated with significant impacts on emotional wellbeing, daily functioning, and social relationships.

Health benefit

8.14. The Committee noted the proposed treatment paradigm provided in the supplier's application and considered the position of alectinib to be appropriate. Members noted funding decisions relating to perioperative immunotherapy regimens for the treatment of NSCLC would not impact the alectinib treatment pathway as defined in the supplier's algorithm.

8.15. The Committee discussed lung cancer scoring systems (eg. ninth edition American Joint Committee on Cancer) and considered this application to regard individuals with tumours four cm or larger, as well as those with tumours of any size accompanied by positive lymph nodes.

8.16. The Committee noted the ALINA study as key evidence, an open-label phase III randomised controlled trial investigating alectinib against platinum-based chemotherapy in adults with completely resected ALK-positive NSCLC (stage IB-IIIA). The alectinib arm comprised 130 individuals receiving alectinib (600 mg, twice daily) until disease progression, unacceptable toxicity, or receiving 24 months of treatment. The chemotherapy arm consisted of 127 individuals who received four 21-day cycles of platinum-based chemotherapy, and members considered the regimens to be comparable to standard practice in New Zealand. The Committee noted that all study participants were required to be eligible to receive platinum-based chemotherapy, with an ECOG performance score of 0-1. The primary endpoint was disease-free survival (DFS).

8.16.1. The Committee noted the findings of Wu et al. N Eng J Med. 2024:390:1265-76, which reported the findings of a pre-specified interim analysis of the ALINA study. The median follow-up was 27.8 months in the alectinib arm, and 28.4 months in the chemotherapy arm.

8.16.1.1. The Committee reviewed the methodology regarding statistical analysis and considered it to be appropriate.

8.16.1.2. The Committee reviewed the demographic and clinical characterises of trial participants and considered the study group to be comparable with the general ALK-positive NSCLC population.

8.16.1.3. The Committee reviewed the safety data and considered the adverse-event profile of alectinib to be consistent with that associated with its use in the advanced setting (ALEX trial; Peters et al. N Engl J Med. 2017;377:829-838). The Committee noted a higher proportion experiencing increased creatine kinase, constipation, and elevations in liver function tests (aspartate aminotransferase, alanine aminotransferase, and bilirubin). Cytopenia and nausea occurred more frequently in the chemotherapy arm. The median duration on treatment with alectinib was 23.9 months and 2.1 months with chemotherapy. Members considered differences in treatment-exposure duration between arms must be considered when interpreting the safety data

from the ALINA study. The Committee noted that serious adverse events occurred in 13.3% of the alectinib arm and 8.3% of the chemotherapy arm. The proportion of people with an adverse event that led to treatment discontinuation was lower in the alectinib arm (5.5%) than in the chemotherapy arm (12.5%). The proportion with a dose reduction was higher in the alectinib arm (25.8%) than in the chemotherapy arm (10%).

8.16.1.4. The Committee noted that, at the time of the pre-specified interim analysis, median DFS in the intention-to-treat (ITT) population had not been reached with alectinib; in the chemotherapy arm, the median was 41.3 months. The hazard ratio for disease recurrence or death was 0.24 (95% CI: 0.13-0.43) favouring alectinib. The proportion of participants in DFS at 2 years was 93.6% for alectinib and 63.7% for the chemotherapy arm. The proportion of participants in DFS at 3 years was 88.7% for alectinib and 54.0% for the chemotherapy arm.

8.16.1.5. The Committee noted the central nervous system (CNS) DFS analysis at 24 months, which was 98.4% (95% CI: 96.1-100) in the alectinib arm and 85.8% (95% CI: 78.8-92.8) in the chemotherapy arm. The Committee noted that CNS assessments were performed at the discretion of the investigator and considered this could be a source of bias. The Committee noted the brain was the most common site of disease recurrence in the ITT population.

8.16.1.6. The Committee considered the data from the interim analysis of ALINA to be immature, in a setting characterised by a relatively prolonged disease course. The Committee discussed the importance of long-term data for targeted adjuvant therapies, and acknowledged the general concern of treatment delaying, not reducing recurrence.

8.16.2. The Committee noted an updated analysis reported in a conference abstract (Dziadziuszko et al. Annuls of Oncol. 2025;36:S971-2), where the median follow up in the ITT population was 47 and 48 months in the alectinib and chemotherapy arms, respectively. The overall survival data was immature at the time of the analysis, and the Committee considered the analysis to be relatively early in the context of resectable NSCLC.

8.16.2.1. Members noted the proportion of participants in DFS at 48 months was 75.5% in the alectinib arm and 47% in the chemotherapy arm. The proportion of participants in DFS at 60 months was 64.8% in the alectinib arm and 42.1% in the chemotherapy arm.

8.16.2.2. The Committee considered there was no obvious waning of treatment effect in the alectinib arm but reiterated the need for longer follow-up.

8.16.3. The Committee noted a conference abstract reporting an exploratory analysis of health-related quality of life from the ALINA study (Nishio et al. J Clin Oncol. 2024;42).

8.16.4. Overall, the Committee considered that the ALINA study demonstrated a large and consistent treatment effect for alectinib across the ITT population and pre-specified subgroups. The Committee regarded ALINA as providing evidence of moderate to high quality. Members noted that the study was open label, with no blinding of participants or clinicians. The primary endpoint was investigator assessed, which was considered to introduce a risk of detection bias. The Committee noted the high rate of treatment adherence and considered the risk of attrition bias to be low.

8.16.5. The Committee discussed the health benefits associated with alectinib and considered there to be a statistically and clinically significant improvement in DFS, including in CNS-DFS, in a safe, chemotherapy-free, oral regimen.

8.16.5.1. The Committee acknowledged the ongoing research questions associated with alectinib as an ALK-tyrosine kinase inhibitor (TKI), including the optimal duration of treatment and the potential use of sequential chemotherapy with an ALK-TKI.

8.16.6. The Committee discussed the extent to which DFS correlates with overall survival in ALK-positive NSCLC. Members acknowledged there is uncertainty and noted the challenges in obtaining mature overall survival data in this setting. The Committee considered that the correlation remains unclear, but that it is reasonable to assume a moderate relationship. The Committee noted that analyses with a median follow-up of at least five years may provide greater confidence.

8.16.7. The Committee discussed the generalisability of the ALINA trial to the New Zealand context and considered the comparator chemotherapy arm to be appropriate. The Committee considered that identifying eligible patients would not be a problem in NZ, noting ALK-testing is already standard practice in NZ in the advanced NSCLC setting. The Committee was aware that ALK-testing was standard practice in early NSCLC in several centres, but considered Pharmac could explore this further with the LOSIG.

8.17. The Committee considered the implementation of alectinib within the current treatment pathway in New Zealand to be feasible. The Committee noted that alectinib spares resource use associated with infusions but requires two years of treatment with associated monitoring costs, compared with chemotherapy, which is typically administered for approximately three months.

8.18. The Committee considered treatment with alectinib to be appropriate for community models of care.

Suitability

8.19. The Committee noted alectinib is an oral treatment, which is convenient for people, but requires up to two years of therapy, compared with around three months of adjuvant chemotherapy in current practice.

Cost and savings

8.20. The Committee considered the possible number of individuals who would be eligible for NSCLC and considered it reasonable to assume up to 0.5% of all NSCLC would be potentially eligible. In making this assumption, the Committee:

8.20.1. agreed with previous advice noting that it was reasonable to assume 5% of all NSCLC is ALK positive (2019-04 CaTSoP Record)

8.20.2. noted the resection data from the Lung Cancer Quality Improvement Monitoring report (Te Aho o Te Kahu. 2025), and considered it reasonable to assume that approximately 20% of all NSCLC is resectable in New Zealand

8.20.3. the Committee noted that approximately 50% of resected lung cancers are <4 cm and N0 and considered it would be reasonable to assume that approximately 50% of resected NSCLC would therefore be eligible for an adjuvant regimen

8.20.4. the Committee considered that some individuals may receive alectinib that would not have received chemotherapy. This was because there are expected

to be more benefits to alectinib treatment that would outweigh the risks associated. The Committee considered that 90% of people with resected ALK-positive NSCLC who are stage-eligible would currently receive adjuvant chemotherapy. It noted that the remaining 10% would not receive treatment where the risks associated with chemotherapy outweigh the expected benefits. The Committee considered this would equate to approximately 0.45% of all people with NSCLC who would receive chemotherapy (individuals with resected ALK-positive disease receiving treatment with adjuvant chemotherapy).

8.20.5. the Committee discussed the potential for sequential treatment with both chemotherapy and alectinib. It was acknowledged the lack of evidence in this setting and considered that, in practice, some individuals with higher-risk tumours, such as stage IIB to IIIA, may be considered for receiving chemotherapy followed by alectinib. The Committee considered it reasonable to assume that 50% of those with 'stage-eligible' resected tumours would receive chemotherapy. Members noted that the use of this sequential treatment approach remains an area of ongoing research.

8.20.6. the Committee noted that alectinib is already funded in the advanced setting. Members considered that, if access were extended to the adjuvant setting, individuals who experience recurrence following adjuvant alectinib would be expected to be re-treated with alectinib, unless progression occurred while receiving adjuvant alectinib

- the Committee discussed the anticipated medical resource use associated with adjuvant alectinib during the first 3 months of treatment with alectinib:
- an individual would require clinic reviews at baseline, six weeks, and three months between months 3-24,
- an individual would require three monthly medical oncology and radiological assessments beyond 24 months,
- individuals will be monitored under respiratory services with approximately six-monthly scans and reviews.
- individuals with progressive disease will require three-monthly medical oncology clinic assessments and radiological assessments.

8.21. The Committee agreed with its previous advice regarding medical resource use associated with adjuvant chemotherapy in NSCLC (2025-11 CTAC record).

8.22. The Committee considered that alectinib treatment was likely to result in improvement or maintenance of health-related quality of life. However, the Committee considered that the data reported in the ALINA trial was short-term and may not adequately pick up on adverse events.

8.23. The Committee considered that ALK testing was standard practice and that the funding of alectinib would be unlikely to result in additional diagnostic-related costs.

Funding criteria

8.24. The Committee discussed potential funding criteria related to an individual's ECOG performance status, noting that the ALINA trial recruited participants with an ECOG performance status of 0 or 1. Members considered that there may be differences in the treatment effect of alectinib in people with an ECOG performance status greater than 1 compared with those with a status of 0 to 1, but considered that any such difference would be smaller than that observed with adjuvant chemotherapy.

8.25. The Committee considered that, in the adjuvant setting, an individual's ECOG performance status is likely to be influenced by recovery from surgery and pre-existing comorbidities, whereas in the advanced setting it is more likely to be driven by disease burden.

8.26. The Committee noted that National Comprehensive Cancer Network guidelines do not specify ECOG performance status criteria.

8.27. The Committee considered that omitting a criterion relating to an individual's ECOG performance status and leaving this to the discretion of the treating clinician, would not be unreasonable.

8.28. The Committee considered that the below summarises its interpretation of the most appropriate PICO (population, intervention, comparator, outcomes) information for alectinib if it were to be funded in New Zealand for the adjuvant treatment of resected ALK positive NSCLC. This PICO captures key clinical aspects of the proposal and may be used to frame any future economic assessment by Pharmac staff. This PICO is based on the Committee's assessment at this time and may differ from that requested by the applicant. The PICO may change based on new information, additional clinical advice, or further analysis by Pharmac staff.

Population	Patients with completely resected ALK-positive, non-small cell lung cancer (tumour ≥4 cm or node positive)
Intervention	600mg of alectinib twice daily for a maximum of 2 years, or until disease recurrence or unmanageable toxicity. Patients with underlying severe hepatic impairment should receive a reduced dose of 450 mg twice daily In the key trial median time on treatment was 23.9 months and median dose intensity was 99%
Comparator(s)	Up to four three-week cycles of platinum-based chemotherapy (median trial duration of treatment 2.1 months and median dose intensity of 100%).
Outcome(s)	Increased disease-free survival, with a moderate likelihood of improved overall-survival - Wu et al. NEJM. 2024;390:1265-1276
Table definitions: Population, the target population for the pharmaceutical; Intervention, details of the intervention pharmaceutical; Comparator, details the therapy(s) that the patient population would receive currently (status quo – including best supportive care); Outcomes, details the key therapeutic outcome(s) and source of outcome data.	

9. Durvalumab for limited-stage small cell lung cancer (LS-SCLC)

Application

9.1. The Committee reviewed the application for durvalumab for the treatment of limited-stage, small cell lung cancer.

Recommendation

9.2. The Committee **recommended** that durvalumab for the treatment of limited-stage, small cell lung cancer be funded with a **high priority** within the context of treatment of malignancy subject to the following Special Authority criteria:

> **Initial approval – (limited-stage small cell lung cancer)** Applications from any relevant practitioner. Approvals valid for 4 months.

All of the following

1. Patient has limited-stage small cell lung cancer; and
2. Patient is being treated with radical intent; and
3. The patient has received platinum based chemoradiation therapy; and
4. The disease has not progressed following platinum based chemoradiation therapy; and
5. The patient had ECOG performance status 0-1 before chemotherapy commences; and
6. Treatment must not exceed 24 months in total from initial dose; and
7. Treatment must not continue beyond disease progression.

Renewal approval – (limited-stage small cell lung cancer) Applications from any relevant practitioner. Approvals valid for 4 months.

1. No evidence of disease progression; and
2. Treatment with durvalumab is to cease after a total duration of 24 months from commencement

9.3. In making this recommendation the Committee considered:

- the high health need of individuals with limited-stage small cell lung cancer
- Māori are disproportionately affected by lung cancer
- clinically meaningful health benefit of improved overall survival
- additional health resource would be required for implementation.

Discussion

Māori impact

9.4. The Committee discussed the impact of funding durvalumab for the treatment of limited-stage, small cell lung cancer on Māori health areas of focus | Hauora Arotahi and Māori health outcomes. The Committee noted:

9.4.1. Treatment of lung cancer is a Hauora Arotahi (Pharmac Māori Health Areas of Focus).

9.4.2. Whilst small cell lung cancer is associated with smoking, Māori patients were 1.55 times more likely to have small cell lung cancer than non-Māori when adjusted for smoking status (Lawrenson et al. NZ Med J. 2018;131:13-23).

9.4.3. A 2023 study reported Māori versus non-Māori ethnicity was not associated with survival for small cell lung cancer (adjusted odds ratio: 1.19; 95% CI: 0.82 - 1.71) (Lawrenson et al.ajo.2023;4:1-9).

Populations with high health needs

9.5. The Committee discussed the health need(s) of individuals with limited-stage, small cell lung cancer among Māori, Pacific peoples, disabled peoples including tāngata whaikaha Māori, and other populations identified by the Government Policy Statement on Health 2024-2027 to have high health needs. The Committee discussed the impact of funding durvalumab and noted:

9.5.1. Individuals from higher deprivation quintiles are associated with increased lung cancer incidences (The State of Cancer in New Zealand 2020, Te Aho O Te Kahu).

9.5.2. Pacific peoples have higher incidence of lung cancer and mortality rates compared with other ethnic groups (The State of Cancer in New Zealand 2020, Te Aho O Te Kahu).

Background

9.6. The Committee considered durvalumab is currently funded for consolidation treatment in stage III non-small cell lung cancer (NSCLC) following chemoradiation.

Health need

9.7. The Committee noted the health need of individuals with extensive-stage (ES)-small cell lung cancer (SCLC) was previously discussed by PTAC in May 2025 who recommended durvalumab be funded with a high priority.

9.8. The Committee considered limited-stage (LS)-SCLC is associated with significant impacts on individuals' quality of life. The Committee noted a study on quality of life, reported fatigue and shortness of breath were the most impactful symptoms that affected individuals with LS-SCLC, as well as facing the long-term physical effects of treatment, financial implications, and emotional toll of an uncertain prognosis (Bebb et al. Pulm Ther. 2023;9:435–50).

9.9. The Committee noted a study of SCLC patients and caregivers reported living with SCLC had a high personal and psychologic burden among caregivers, whose duties consumed much of their time. Caregivers observed similar symptoms and impacts of SCLC to those reported by patients (Bebb et al. 2023).

9.10. The Committee noted that whilst SCLC is associated with smoking, Māori patients were 1.55 times more likely to have SCLC than non-Māori when adjusted for smoking status (Lawrenson et al. NZ Med J. 2018;131:13-23). The Committee noted that Māori also have poorer survival outcomes from lung cancer overall than non-Māori (The State of Cancer in New Zealand 2020, Te Aho O Te Kahu).

9.11. The Committee noted a 2023 study reported there was no significant evidence of disparities in patterns of care and outcomes between Māori and non-Māori with SCLC. Māori versus non-Māori ethnicity was not associated with survival (adjusted odds ratio (OR): 1.19; 95% CI: 0.82 - 1.71) (Lawrenson et al.ajo.2023;4:1-9).

9.12. The Committee noted a 2024 Waikato District Health Board study on lung cancer patients presenting through emergency department (ED) visits, reported Māori were 1.27 times more likely than non-Māori to be diagnosed after attending the ED. Patients attending the ED were less likely to survive 12 months than those without ED visits (OR 0.42), and those with two or more ED visits were even less likely to survive 12 months (OR 0.33) (Lawrenson et al. N Z Med J. 2024;137:14-24).

9.13. The Committee noted individuals from higher deprivation quintiles are associated with increased lung cancer incidences (The State of Cancer in New Zealand 2020, Te Aho O Te Kahu).

9.14. The Committee noted that Pacific peoples have a higher incidence of lung cancer and mortality rates compared with other ethnic groups (The State of Cancer in New Zealand 2020, Te Aho O Te Kahu).

9.15. The Committee considered LS- SCLC accounts for approximately 30% of all people with SCLC. The Committee considered SCLC is treated according to stage, with patients referred to multidisciplinary meetings to determine the most appropriate care pathway.

9.16. The Committee considered the majority of those with LS-SCLC are treated with curative intent. The treatment comprises concurrent chemoradiation with a platinum-based chemotherapy, most often cisplatin or alternatively carboplatin, and etoposide with radiation, followed by active surveillance until relapse. The Committee considered approximately 90% have a response to treatment.

9.17. The Committee considered that whilst the standard treatment was concurrent chemoradiation, which is considered superior, a minority would receive sequential

chemoradiation. The Committee considered sequential chemoradiation was typically in individuals who had a poor performance status and anecdotally for logistical reasons, used in some rural centres. The Committee considered this could be due to reduced access to timely radiation. The Committee considered that sequential chemoradiation could still be considered a curative intent treatment.

9.18. The Committee considered prophylactic cranial irradiation (PCI) is considered for all patients.

9.19. The Committee considered there was a high rate of relapse during the active surveillance phase. The Committee noted a study that reported most individuals experience disease relapse within two years after starting treatment (Cheng et al. N Engl J Med. 2024 10;391:1313-27).

Health benefit

9.20. The Committee noted ADRIATIC, a phase III, double-blind, randomised, placebo-controlled trial that compared durvalumab monotherapy (n=264) or durvalumab (plus tremelimumab for four doses, n=200) or placebo (n=266) every four weeks for up to 24 months.

9.20.1. The Committee noted Cheng et al. N Engl J Med. 2024 10;391:1313-27 that reported the following results:

- Overall survival (OS): Durvalumab monotherapy therapy led to significantly longer OS than placebo (median, 55.9 months [95% confidence interval {CI}, 37.3 to not reached] compared to 33.4 months [95% CI, 25.5 to 39.9]; hazard ratio (HR) for death, 0.73; 98.321% CI, 0.54 to 0.98; P=0.01). OS at 3 years was 56.5% with adjuvant durvalumab therapy compared to 47.6% in the placebo group
- Progression free survival (PFS): Durvalumab had a median PFS of 16.6 months (95% CI, 10.2 to 28.2) compared to 9.2 months (95% CI, 7.4 to 12.9) for the placebo group. HR for progression or death of 0.76; 97.1, 95% CI, 0.59 to 0.98; P=0.02.

9.20.2. The Committee noted Spigel et al. JCO.2024.42.17_suppl.LBA5 that reported the following results:

- Median (range) duration of follow-up for OS and PFS was 37.2 (0.1–60.9) and 27.6 (0.0–55.8) months, respectively.
- OS was significantly improved with durvalumab compared to placebo (HR 0.73 [95% CI 0.57–0.93]; p=0.0104; median OS 55.9 [95% CI 37.3 – not estimable] compared to 33.4 [25.5–39.9] months respectively). 24-month OS rate was 68.0% for durvalumab compared to 58.5% in the placebo group; 36-month OS rate 56.5% vs 47.6% respectively.
- PFS was also significantly improved with durvalumab compared to placebo (HR 0.76 [95% CI 0.61–0.95]; p=0.0161). Median PFS for durvalumab was 16.6 months [95% CI 10.2–28.2] compared 9.2 months [7.4–12.9] placebo. The 18-month PFS rate was 48.8% vs 36.1%, and the 24-month PFS rate 46.2% vs 34.2% respectively.

9.21. The Committee noted there was a protocol amendment that changed the randomisation to 1:1 durvalumab monotherapy and placebo. The dual therapy group remained blinded.

9.22. The Committee considered that whilst individuals included in the trial were generally fitter than those treated in New Zealand the results were generalisable to the New Zealand population.

9.23. The Committee considered the baseline characteristics were comparable between arms.

9.24. The Committee noted that approximately 65% received cisplatin compared to 35% carboplatin. The Committee considered this was similar to the New Zealand setting where the proportion would be 60%:40% respectively. The Committee considered most received PCI which is consistent with the New Zealand setting.

9.25. The Committee noted the OS and PFS results from the placebo group were greater than those previously observed in historical controls, however the Committee considered that the historical control results predated advancements in chemoradiation techniques, and subsequent therapies were not as efficacious.

9.26. The Committee noted that a higher proportion in the durvalumab group experienced adverse events that led to discontinuation, including pneumonitis or radiation pneumonitis that led to discontinuation in 8.8% compared to 3% in the placebo arm.

9.27. The Committee considered the data is limited to an interim analysis, and the final analysis data is not available. The Committee considered the follow up period was relatively short, and there was limited data available on subgroups.

9.28. The Committee considered overall the trial evidence was high quality and reported a statistically significant and clinically meaningful OS and PFS benefit compared to active surveillance.

Suitability

9.29. The Committee considered while the infusion time is relatively short (60 minutes) it would still represent an additional treatment burden to the individual, who would be required to travel to an infusion centre for treatment compared to active surveillance.

Cost and savings

9.30. The Committee considered individuals who relapse in the LS-SCLC setting are usually treated in accordance with the ES-SCLC treatment paradigms unless progression was within three months of completing chemoradiation.

9.31. The Committee considered approximately 50% of individuals who relapsed following chemoradiation received a second line chemotherapy. The Committee considered this was typically carboplatin etoposide treatment.

9.32. The Committee considered the majority of individuals who relapsed in the ADRIATIC trial received a platinum doublet therapy. The Committee noted that palliative immunotherapies are not funded in New Zealand. The Committee considered immunotherapy (including atezolizumab or tarlatamab) provided an approximately two-month OS benefit compared with chemotherapy alone.

9.33. The Committee considered approximately 80% of individuals in the eligible population would be well enough for durvalumab treatment. The Committee considered an individual's Eastern Cooperative Oncology Group (ECOG) Performance Status score would improve with chemoradiation.

9.34. The Committee considered approximately 70% of individuals with LS-SCLC are treated with chemoradiation.

9.35. The Committee considered approximately 90% of people respond to chemoradiation and do not progress while on treatment.

9.36. The Committee considered that patients would receive durvalumab within 42 days of receiving concurrent chemoradiation therapy and no prevalent population beyond this would be expected.

9.37. The Committee considered if durvalumab treatment were funded, approximately 80% of people in year one would receive treatment, which would increase to 100% by year two.

9.38. The Committee considered that these individuals would already be receiving care from medical oncology services and clinicians would support a therapy that offers survival benefit. The Committee considered that there was no alternative treatment in this setting and considered that the rate limiting factor to uptake would be implementation.

9.39. The Committee considered that the greatest resource impact would arise from the additional two years of monitoring required for individuals receiving treatment who would not otherwise have been under the care of medical oncology services.

9.40. The Committee considered it reasonable to assume that the health-related quality of life (HRQoL) observed in ES-SCLC would be representative of the HRQoL experienced by individuals with LS-SCLC who relapse following chemoradiation.

9.41. The Committee considered it was reasonable to extrapolate the outcomes from the ADRIATIC study to individuals treated with sequential chemoradiation.

9.42. The Committee considered progression typically occurs within two years, and that new recurrences after five years are very rare.

Funding criteria

9.43. The Committee considered there should be no restriction on the type of chemoradiation (concurrent or sequential) that individuals receive.

Summary for assessment

9.44. The Committee considered that the below summarises its interpretation of the most appropriate PICO (population, intervention, comparator, outcomes) information for durvalumab if it were to be funded in New Zealand for LS-SCLC which has not progressed following platinum-based chemoradiation therapy. This PICO captures key clinical aspects of the proposal and may be used to frame any future economic assessment by Pharmac staff. This PICO is based on the Committee's assessment at this time and may differ from that requested by the applicant. The PICO may change based on new information, additional clinical advice, or further analysis by Pharmac staff.

Population	Individuals with limited-stage small cell lung cancer which has not progressed following platinum-based chemoradiation therapy (CRT) (concurrent or sequential CRT, and optional prophylactic cranial irradiation (PCI)) and have an ECOG PS of 0-1.
Intervention	Durvalumab: 1500mg every 4 weeks for maximum 24 months or until disease progression or unacceptable toxicity
Comparator(s)	Active surveillance
Outcome(s)	- Improved overall survival, per the ADRIATIC trial (ITT population) - Improved health-related quality of life as a result of longer time in progression-free survival, (PFS), per the ADRIATIC trial (ITT population).
Table definitions: Population, the target population for the pharmaceutical; Intervention, details of the intervention pharmaceutical; Comparator, details the therapy(s) that the patient population would receive currently (status quo – including best supportive care); Outcomes, details the key therapeutic outcome(s) and source of outcome data.	

10. Inavolisib for locally advanced or metastatic breast cancer, PIK3CA mutated, HR+ HER2-

Observers

10.1. Dr James Le Fevre, Chair of the National Clinical Practice Committee (NCPC) at Health New Zealand and Anita Fitzgerald, Principal Advisor Health Technology Assessment & Horizon Scanning / NCPC at Health New Zealand attended this item as Observers, to share context regarding the diagnostic test component of this proposal. As Observers they attended in-line with the Specialist Advisory Committee Terms of Reference and left the meeting before the recommendation was made.

Application

10.2. The Committee reviewed the application for inavolisib (in combination with palbociclib and endocrine therapy) for the treatment of PIK3CA-mutated, hormone receptor (HR)-positive, human epidermal growth factor receptor 2 negative (HER2-) locally advanced or metastatic breast cancer, following recurrence on/after adjuvant therapy

10.3. The Committee took into account, where applicable, Pharmac's relevant decision-making framework when considering this agenda item.

Recommendation

10.4. The Committee **recommended** that inavolisib (in combination with palbociclib and endocrine therapy) for the treatment of HR-positive, HER2-negative locally advanced or metastatic breast cancer with a *PIK3CA*-mutation, following recurrence on/after adjuvant therapy be funded with a **medium priority** within the context of treatments of malignancy subject to the following Special Authority criteria:

> **Initial application** – (breast cancer, metastatic) Applications from any relevant practitioner.
> Approvals valid for 6 months for applications meeting the following criteria:
> All of the following:
> 1. Patient has unresectable, inoperable locally advanced or metastatic breast cancer that is confirmed as hormone receptor positive and HER2- negative; and
> 2. Patient has a confirmed PIK3CA gene mutation; and
> 3. Patients with hormone-receptor positive breast cancer must have received at least one line of adjuvant endocrine therapy and had disease progression during therapy, or within 12 months of completing adjuvant endocrine therapy with an aromatase inhibitor or tamoxifen; and
> 4. Patient has an ECOG score of 0-1; and
> 5. Must be received in combination with palbociclib and fulvestrant; and
> 6. Patient has not received prior systemic therapy in the metastatic setting; and
> 7. Inavolisib to be discontinued at disease progression.
>
> **Renewal application** – (breast cancer, metastatic) Applications from any relevant practitioner.
> Approvals valid for 6 months for applications meeting the following criteria:
> Both:
> 1. No evidence of disease progression; and
> 2. Inavolisib to be discontinued at disease progression

10.5. In making this recommendation the Committee considered:

- the poorer health outcomes of those with *PIK3CA* mutated breast cancer
- the need for an additional line of therapy for those with *PIK3CA* mutated breast cancer who tend to be younger
- the concern on the equitable access to the treatment given the lack of information on the health benefit and adverse effect profile for those with type two diabetes
- the concern regarding the equitable access to genetic testing across New Zealand.

Discussion

Māori impact

10.6. The Committee discussed the impact of funding inavolisib for the treatment of *PIK3CA*-mutated, HR-positive, HER2-negative locally advanced or metastatic breast cancer on Māori health areas of focus | Hauora Arotahi and Māori health outcomes. The Committee considered:

10.6.1. the treatment of breast cancer is a Hauora Arotahi (Pharmac Māori Health Areas of Focus)

10.6.2. previous CTAC discussion in November 2025 on genetic testing in New Zealand. The Committee considered that similarly to *BRCA* testing reference databases were predominantly compromised of individuals from European descent and it was more likely that individuals not from European decent, including Māori would have variants of unknown significance which would impact the diagnostic accuracy of testing

10.6.3. the lack of New Zealand specific data on rates of *PIK3CA* mutations in Māori compared to non-Māori

10.6.4. the Committee noted there is a higher incidence of breast cancer in Māori, that is diagnosed at a later stage. The Committee note that Māori have worse health outcomes compared to New Zealand Europeans

10.6.5. in addition, the Committee noted that Māori women are approximately 5 years younger at diagnosis than non-Māori women.

Populations with high health needs

10.7. The Committee discussed the health need(s) of *PIK3CA*-mutated, HR-positive, HER2-negative locally advanced or metastatic breast cancer among Māori, Pacific peoples, disabled peoples including tāngata whaikaha Māori, and other populations identified by the Government Policy Statement on Health 2024-2027 to have high health needs. The Committee discussed the impact of funding inavolisib and considered:

10.7.1. previous CTAC discussion in November 2025 on genetic testing in New Zealand. The Committee considered that similarly to *BRCA* testing reference databases were predominantly compromised of individuals from European descent and it was more likely that individuals not from European decent, including Pacific peoples would have variants of unknown significance which would impact the diagnostic accuracy of testing

10.7.2. *PIK3CA* mutation testing is variable across regional centres which may provide inequitable access to testing.

Health need

10.8. The Committee noted the health need of individuals with HER2-, HR+ locally advanced, metastatic breast cancer was recently discussed by CTAC in November 2025.

10.9. The Committee considered that the reported median prevalence of *PIK3CA* mutation was 36% (range: 13.3% to 61.5%). The Committee considered there was there was some evidence that there was concordance between liquid and solid tumour biopsy. The Committee considered the prevalence of specific *PIK3CA* mutations varied between studies, and that there was a strong European bias in the populations included in the studies (Anderson et al. Int J Breast Cancer. ;2020:3759179)

10.10. The Committee noted Schagerholm et al. Sci Rep. 2024;12542 that reported a high concordance between primary tumours that expressed a *PIK3CA* mutation and relapsed lesions. The Committee considered a small percentage (~10%) that did not have a *PIK3CA* mutation gained a mutation following relapse. The Committee noted the study reported tumours with a *PIK3CA* mutation were associated with nodal involvement, and therefore individuals were treated with more extensive surgery and had a higher likelihood of chemotherapy.

10.11. The Committee considered there was some evidence to support expression of a *PIK3CA* mutation may be a poor prognostic factor in individuals whose tumours are endocrine therapy (ET) resistant. The Committee noted there was a small retrospective cohort study in individuals with ET resistant ER+/HER2- metastatic breast cancer (Diece et al. Eur J Cancer. 2026:234:116194). The Committee considered that individuals with a *PIK3CA* mutation had a worse progression free survival (PFS) compared to those without. However, the Committee considered that there was no evidence to suggest that a *PIK3CA* mutation is predictive of ET resistance.

10.12. The Committee considered anecdotal evidence that *PIK3CA* mutation testing is performed in Auckland and Christchurch. The Committee considered that this testing would be by request, and that there would be variability across regional centres for testing. The Committee considered this may provide inequitable access to testing.

10.13. The Committee noted CTAC's comments in November 2025 on genetic testing in New Zealand. The Committee considered that similarly to *BRCA* testing, reference databases were predominantly compromised of individuals from European descent, and it was more likely that individuals not from European decent, including Māori and Pacific peoples would have variants of unknown significance which would impact the diagnostic accuracy of testing. The Committee considered in general there are differences in mutations across ethnic groups, however the differences in pathogenic variants of interest will not be clearly delineated until a larger testing consortium is established that includes New Zealand data.

Health benefit

10.14. The Committee noted INAVO120, a phase III, double-blind, randomised trial that compared inavolisib plus palbociclib and fulvestrant with placebo plus palbociclib and fulvestrant in 325 individuals. Trial results were published in the following publications:

- Turner et al. NEJM 2024;391;1558-96
- Jhaveri et al. N Engl J Med 2025;393:151-161

10.14.1. The Committee noted at the following results for INAVO120 were reported at a median of follow up of 21.3 months inavolisib, and 21.5 months placebo:

- Median PFS: 15.0 months (95% confidence interval [CI], 11.3 to 20.5) inavolisib vs 7.3 months (95% CI, 5.6 to 9.3) placebo (hazard ratio (HR) for disease progression or death, 0.43; 95% CI, 0.32 to 0.59; P<0.001).
- Grade 3 or 4 adverse events (AE) (inavolisib vs placebo respectively):
  - Neutropenia: 80.2% vs 78.4%
  - Hyperglycaemia: 5.6% vs 0%,
  - Stomatitis or mucosal inflammation: 5.6% vs 0%
  - Diarrhoea: 3.7% vs 0%.

10.14.2. The Committee noted that whilst PFS met statistical significance, the overall survival (OS) did not. However, the Committee noted the publication was an interim analysis for OS.

10.14.3. The Committee noted at the following results for INAVO120 were reported at a median follow up of 34.2 months inavolisib, and 32.3 months placebo:

- PFS: 17.2 months with inavolisib vs 7.3 months placebo (stratified HR for disease progression or death, 0.42; 95% CI, 0.32 to 0.55).
- Final OS analysis: Median OS was 34.0 months (95% CI, 28.4 to 44.8) with inavolisib and 27.0 months (95% CI, 22.8 to 38.7) with placebo (HR for death, 0.67; 95% CI, 0.48 to 0.94; P = 0.02 [prespecified boundary for statistical significance, P<0.0469]).

10.15. The Committee noted that at the final analysis the OS was significant.

10.16. The Committee considered inavolisib treatment was frequently associated with stomatitis and mucosal inflammation, as well as hyperglycaemia.

10.17. The Committee noted that individuals with either type one or type two diabetes were excluded from the trial.

10.18. The Committee considered that recruitment to the trial took longer than expected and therefore the overall numbers included were lower.

10.19. The Committee considered that the majority of individuals included had relatively high-volume disease on progression, with multiple sites of metastases.

10.20. The Committee considered approximately one third of individuals included in the trial had ET resistance during adjuvant treatment, with approximately two thirds following adjuvant treatment.

10.21. The Committee considered that individuals who received tamoxifen monotherapy as a previous treatment had the greatest gain in PFS compared to those who had received aromatase inhibitors either as a monotherapy or in combination with tamoxifen.

10.21.1. The Committee considered that individuals who had received tamoxifen monotherapy and would also receive a gonadotropin-releasing hormone (GNRH) analogue when commencing inavolisib treatment and therefore would effectively be moving from mono to tandem therapy.

10.22. The Committee considered there were revisions to the statistical analysis plan between the results being published. The Committee considered this amendment was a clarification rather than a protocol change and did not affect the results.

10.23. The Committee considered that whilst overall the data was high quality and generalisable to the New Zealand population, as the trial excluded those with diabetes it would be challenging to extrapolate the data to these individuals. The Committee also considered the average weight of those included in the trial is lower than the New Zealand average.

10.24. Th Committee considered it would welcome funding applications for other *PI3K* inhibitors including alpelisib.

Suitability

10.25. The Committee noted that individuals with type one or two diabetes were excluded from the trial, and therefore considered there is a lack of safety and health benefit evidence in this population.

10.26. The Committee considered that oncologists routinely manage diabetes that is related to oncology treatments. This can be clinically complex and require additional oncology appointments.

Cost and savings

10.27. The Committee considered up to 50% of people with HR+/HER2- metastatic breast cancer have a *PIK3CA* mutation.

10.28. The Committee considered that approximately 100-150 individuals may be eligible for inavolisib treatment.

10.29. The Committee considered uptake would include approximately 90-100% of the eligible population.

10.30. The Committee considered that individuals must be post-menopausal either naturally or via GNRH analogue. This may represent an additional treatment cost.

10.31. The Committee noted that the *PIK3CA* mutations eligible for inclusion in the pivotal trial were broader than those included in the current testing panel in New Zealand. The Committee considered that the eligible population in New Zealand may increase if the testing frequency increases, and the panel broadens to include more mutations.

10.32. The Committee considered anecdotal evidence that patients were experiencing higher rates of hyperglycaemia than reported in the trial, and that clinicians were initiating metformin treatment when commencing inavolisib treatment.

10.33. The Committee considered that the mucosal inflammation was treated empirically using a dexamethasone mouthwash and recommended for the first eight weeks of treatment.

10.34. The Committee considered individuals would receive additional routine glucose monitoring during treatment.

10.35. The Committee considered if other cyclin dependent kinase 4/6 (CDK4/6) inhibitors would be appropriate to consider alongside inavolisib treatment. The Committee considered that ribociclib is associated with a higher frequency of gastrointestinal adverse events which would likely compound with the gastrointestinal adverse events observed with inavolisib. Therefore, the Committee considered that whilst the class of CDK4/6 inhibitors may provide similar health benefit, the different agents may provide a significant increase to the toxicity experienced by the individual.

10.36. The Committee considered as the trial was restricted to Eastern Cooperative Oncology Group (ECOG) performance status (PS) 0-1 it was likely that the magnitude of health benefit would be lower in those with an ECOG >1 however there was a lack of data to support use in this population.

10.37. The Committee considered that the number of individuals with an ECOG >2 would be small in this population. The Committee consider this population were well monitored during ET. The Committee considered most of these individuals would be treated with inavolisib if it was available however the duration of treatment would be short. The Committee considered very few of this population would have a poorer ECOG status, and these individuals would currently be treated with ET with a cyclin dependent kinase 4/6 (CDK4/6) inhibitor, or ET monotherapy. Individuals with an acute decline for example visceral crisis would receive chemotherapy.

10.38. The Committee considered most would receive chemotherapy after progression on inavolisib. A small population may receive best supportive care.

Funding criteria

10.39. The Committee considered it was appropriate to restrict treatment to those who had not received a CDK4/6 inhibitor in the adjuvant setting. The Committee considered CDK4/6 inhibitor uptake in the adjuvant setting would be high if funded. The Committee considered that this would therefore increase second line inavolisib treatment.

Summary for assessment

10.40. The Committee considered that the below summarises its interpretation of the most appropriate PICO (population, intervention, comparator, outcomes) information for inavolisib if it were to be funded in New Zealand for *PIK3CA*-mutated, HR-positive, HER2 negative, locally advanced or metastatic breast cancer. This PICO captures key clinical aspects of the proposal and may be used to frame any future economic assessment by Pharmac staff. This PICO is based on the Committee's assessment at this time and may differ from that requested by the applicant. The PICO may change based on new information, additional clinical advice, or further analysis by Pharmac staff.

Population	Adult patients with *PIK3CA*-mutated, hormone receptor (HR)-positive, human epidermal growth factor receptor 2 (HER2)-negative, locally advanced or metastatic breast cancer, following recurrence on or after adjuvant therapy (within 12 months)
Intervention	Inavolisib with palbociclib and endocrine therapy (e.g., fulvestrant) • Inavolisib: 9 mg taken orally once daily until disease progression or unacceptable toxicity. • Palbociclib: 125 mg taken orally once daily for 21 consecutive days followed by 7 days off treatment to comprise a complete cycle of 28 days
Comparator(s)	CDK 4/6 inhibitor (ribociclib, palbociclib) in combination with fulvestrant
Outcome(s)	Per the INOV120 trial • Improved health-related quality of life due to prolonged progression free survival • Improved overall survival
Table definitions: Population, the target population for the pharmaceutical; Intervention, details of the intervention pharmaceutical; Comparator, details the therapy(s) that the patient population would receive currently (status quo – including best supportive care); Outcomes, details the key therapeutic outcome(s) and source of outcome data.	

11. Ibrutinib for mantle cell lymphoma, upfront therapy as alternative to ASCT

Application

11.1. The Committee reviewed the clinician application submitted on behalf of the New Zealand Lymphoma Network for the use of ibrutinib (Imbruvica) for the first-line treatment of mantle cell lymphoma (MCL).

11.2. The Committee took into account, where applicable, Pharmac's relevant decision-making framework when considering this agenda item.

Recommendation

11.3. The Committee **recommended** access to ibrutinib be widened to include the first line treatment of mantle cell lymphoma (with funding of rituximab maintenance extended to three years for this group) with a **high priority**, within the context of treatment of malignancy, subject to the following Special Authority criteria:

> **Initial application** — mantle cell lymphoma (MCL), first-line
> Applications from any relevant practitioner. Approvals valid for 6 months.
> All of the following:
> 1. Individual has mantle cell lymphoma (MCL) requiring first-line therapy; and
> 2. Individual is eligible for autologous stem cell transplant (ASCT), but ibrutinib is intended to be used instead of this procedure and not in addition to ASCT; and
> 3. Ibrutinib is to be used as part of first-line therapy containing R-CHOP/RDHAC/RDHOx/or any other platinum-containing regimen.

Renewal — mantle cell lymphoma (MCL), first-line
Applications from any relevant practitioner. Approvals valid for 9 months.
1. There is no evidence of disease progression; and
2. Ibrutinib is to be used for a maximum duration of two years.

11.4. In making this recommendation, the Committee considered:

11.4.1. there was evidence of likely benefits of ibrutinib compared with autologous stem cell transplant (ASCT) for transplant-eligible MCL

11.4.2. avoiding ASCT entirely for people with transplant-eligible MCL would provide substantial benefits to the individual, family/whanau, and the health system including an opportunity gain for other people waiting for transplantation.

11.5. The Committee considered that if ibrutinib were to be funded for first-line treatment of MCL, the previously recommended funding criteria for ibrutinib/zanubrutinib for relapsed or refractory (R/R) MCL may require amendment to confirm that there is no known resistance to a Bruton tyrosine kinase inhibitor (BTKi) at the time of progression. However, members noted there was a lack of data at this time to inform consideration of subsequent treatment with ibrutinib for MCL (eg for those with disease that is not refractory to ibrutinib, and a window of one year since last receiving ibrutinib).

Discussion

Māori impact

11.6. The Committee discussed the impact of funding ibrutinib for the first-line treatment of MCL on Māori health areas of focus | Hauora Arotahi and Māori health outcomes. The Committee noted there was no evidence to suggest differential incidence for Māori compared with non-Māori, although this does not exclude the possibility of differences.

Populations with high health needs

11.7. The Committee discussed the health need(s) of people with MCL among Māori, Pacific peoples, disabled peoples including tāngata whaikaha Māori, and other populations identified by the Government Policy Statement on Health 2024-2027 to have high health needs.

11.7.1. The Committee discussed the impact of funding first-line ibrutinib for MCL and considered there was no evidence to suggest differential incidence for groups with high health needs compared with other groups, although this does not exclude the possibility of differences.

11.7.2. The Committee considered that there was no identified data on access to ASCT (or its impacts) at the larger treatment centres which perform the procedure for provincial or rural people with MCL. However, the Committee considered that there would be inequities in access for these patients that would be removed by funding ibrutinib first-line for MCL, due to it avoiding the need for ASCT and being used in the community.

11.7.3. The Committee considered that people living in rural or regional areas would experience substantial impacts from ASCT (eg from infection risk and inpatient stays associated with undergoing ASCT) and that less intensive therapy with ibrutinib, as an oral treatment, would offer considerable suitability benefits compared with ASCT.

Background

11.8. The Committee noted that ibrutinib (Imbruvica) and zanubrutinib (Brukinsa) for the treatment of relapsed or refractory (R/R) MCL have both been ranked to Pharmac's Options For Investment (OFI) list.

11.9. The Committee noted that ibrutinib is currently funded with restriction to the treatment of chronic lymphocytic leukaemia (CLL). It has been considered for a range of haematology oncology indications including leukaemia, lymphoma and Waldenstrom's macroglobulinaemia (link to Application Tracker).

Health need

11.10. The Committee noted that the Committee had considered the health need of people with MCL in July 2023 during its consideration of zanubrutinib for R/R MCL (link to record).

11.11. The Committee noted that MCL accounts for about 6% of non-Hodgkin lymphomas (Skarbnik et al. Discov Med. 2013;15:177-87) with an estimated 51 new cases per year in New Zealand (Ministry of Health. Cancer Registry - New Registrations [ICD Code C83.1 - Mantle Cell Lymphoma]. 2023). The Committee noted that MCL is usually diagnosed at an advanced stage in people about 60 years of age (Chandran et al. Leuk Lymphoma. 2012;53:1488-93).

11.12. The Committee noted the MCL International Prognostic Index (MIPI) score grading for MCL, and cellular MIPI (cMIPI) including the Ki-67 cell proliferation index. Members considered that a combination of MIPI/cMIPI score, the presence of blastoid or low-grade cytology, and TP53 mutation can result in 'high risk' MCL biology that responds poorly to chemotherapy and is associated with survival of approximately two years or less.

11.13. The Committee noted the first-line treatment of transplant-eligible MCL includes 4-6 cycles of induction chemotherapy (including high dose cytarabine) followed by ASCT and then followed by two years of rituximab maintenance. Members noted that while available internationally, in New Zealand there is no funded BTKi for this population. The Committee considered that the most used induction chemotherapy regimens for transplant-eligible MCL are the Merryman protocol (alternating BR and HiDAC) and the Nordic protocol (R-Maxi-CHOP/R-ARAC):

11.13.1. Members noted that the longest reported median overall survival (OS) of 12.7 years for transplant-eligible MCL was with the maxi-CHOP regimen (which uses higher doses than R-CHOP and alternated with high dose cytarabine, followed by ASCT) in the Nordic MCL2 trial (Eskelund et al. Br J Haematol. 2016;175:410-8).

11.13.2. Members considered that induction chemotherapy with rituximab-bendamustine, and rituximab-cytarabine (RB/RC) followed by ASCT and followed by rituximab maintenance is standard of care at some North Island treatment centres, for which there is emerging evidence of 3-year progression-free survival and overall survival of 83% and 92%, respectively (Merryman et al. Blood Adv. 2020;4:858-67).

11.14. The Committee noted that ASCT involves the following: apheresis (stem cell harvest), travel to transplant centres, toxicities associated with chemotherapy, inpatient stay, close monitoring of early follow up, time off work for the individual and their family, significant fatigue, and recovery.

11.15. The Committee noted that the use of cytarabine-containing chemotherapy requires hospital admission given that cytarabine involves twice daily dosing. The Committee noted that three hospital admissions are needed during induction, with five days minimum for harvest, then 3-4 weeks in hospital for the ASCT. The Committee

considered that some people choose to decline having an ASCT due to the impact of the procedure on themselves and family and noted that some ASCTs can be limited by difficulty harvesting.

11.16. The Committee noted the current barriers to timely SCT, being long waitlists and limited capacity at the few larger centres which offer SCT (including allogeneic and autologous). The Committee considered that some centres may offer additional chemotherapy cycles to bridge the time and hold disease until transplant can occur.

11.17. The Committee noted that ASCT-related toxicity can be significant and potentially life-threatening, predominantly relating to febrile neutropenia (in approximately half of cases or more), infections and thrombocytopenia. The Committee noted that patients undergoing ASCT require intensive follow-up and blood monitoring, with substantial transfusion support expected in the first 30 days post-ASCT.

11.18. The Committee considered that most people with MCL in this group are well served by standard of care treatments and that their unmet need is from the avoidable toxicity of ASCT, although acknowledged those with high-risk disease have an unmet need due to limited survival gains with standard of care chemotherapy. The Committee considered there is a need for reduced toxicity whilst maintaining very effective treatment, noting the substantial impact of the disease and treatment on family and wider society.

11.19. The Committee noted that transplant-ineligible patents with MCL (a group who are generally older, frailer, and less able to receive intensive chemotherapy) would require separate consideration. The Committee noted Pharmac had not yet received an application for ibrutinib for this group, which would likely include evidence from the SHINE trial (Wang et al. N Engl J Med. 2022;386:2482-64). Members considered that the ECHO study of RB with acalabrutinib may also be relevant if the transplant-ineligible group is considered.

Health benefit

Ibrutinib for 1L MCL

11.20. The Committee noted that ibrutinib is indicated for the treatment of patients with MCL who have received at least one prior therapy (link to Medsafe datasheet). The Committee noted ibrutinib is a well-known BTKi which stops B-cell proliferation, migration and survival. The Committee noted that dosing for MCL is 560mg daily on days one to 19 of each 21-day R-CHOP (rituximab cyclophosphamide doxorubicin vincristine prednisolone) chemotherapy cycle, for a total of 3 cycles followed fixed-duration maintenance ibrutinib (560 mg orally each day for 2 years)

11.21. The Committee noted the proposed algorithm by New Zealand Lymphoma Network and its comment that first-line ibrutinib would represent a significant benefit to patients by reducing exposure to ASCT toxicity, and that ibrutinib would be an alternative to ASCT (ie not used in addition to ASCT).

11.22. The Committee noted the key evidence from TRIANGLE; a phase III, open-label, three arm, randomised (1:1:1), superiority trial in 870 transplant-eligible patients with previously untreated, stage II–IV MCL aged 18–65 years (Dreyling et al. Lancet. 2024;403:2293-306).

11.22.1. The Committee noted that, consistent with the overall lymphoma population, participants were predominantly European; the majority were male; and a good proportion had high-risk disease biology. The Committee noted that this multicentre study included three groups with three pairwise comparisons, and that the standard immunochemotherapy regimens used were R-CHOP, R-

DHAP or R-DHAOx (rituximab, dexamethasone, cytarabine + cisplatin/oxaliplatin).

11.22.2. The Committee noted that treatment included standard immunochemotherapy + ASCT (Group A); standard immunochemotherapy + ASCT + ibrutinib induction and maintenance for two years (Group A+I); and standard immunochemotherapy + ibrutinib (without ASCT; Group I). The Committee noted that TRIANGLE was designed before rituximab maintenance became standard of care, however, that rituximab maintenance for three years was added to trial treatment by protocol amendment and was received by half of all participants, evenly split across groups.

11.22.3. The Committee noted the analysis of the primary outcome for failure-free survival (FFS) included those with stable disease and considered this reasonable given it reflected those whose disease did not respond to induction chemotherapy and would therefore have a poorer prognosis. The Committee noted that after median follow-up of 31 months, 3-year FFS was 72% (67–79) in Group A; 88% (84-92) in Group A+I; and 86% (82–91) in Group I (hazard ratio 1·77 [one-sided 98·3% CI 0–3·76]; one-sided p=0·9979). The Committee noted that the pairwise comparison of A+I vs I for FFS was still ongoing at the time of publication.

11.22.4. The Committee noted the early divergence of Kaplan-Meier curves for FFS suggests ibrutinib deepens the treatment response, and that the treatment effect was durable with a plateau extending beyond two years of maintenance treatment. The Committee noted there were impressive outcomes with ibrutinib for the subset of participants with high-risk MCL who are known to experience poor outcomes with standard of care chemotherapy.

11.22.5. The Committee noted that progression-free survival (PFS) results reported the greatest benefit in the treatment arms containing ibrutinib (Groups A+I and I) compared with ASCT.

11.22.6. The Committee noted that 3-year overall survival (OS) was 86% (82-91) in Group A; 91% (88-95) in Group A+I; and 92% (88-95) in Group I.

11.22.7. The Committee considered these results indicate ASCT can be omitted for this population without compromising efficacy in terms of FFS, and noted there was a non-significant trend to suggest OS is similar between arms.

11.23. The Committee noted that toxicities reported from the TRIANGLE study can be considered across the three treatment phases, with most of the overall toxicity and therefore difference in adverse events being during the ASCT phase:

11.23.1. Induction phase: The Committee noted that there were similar incidence and types of grade 3-5 adverse events (AEs) between Group A and Group I, with slight increases in the incidence of anaemia, cytopenias and the risk of infections with ibrutinib.

11.23.2. ASCT phase: The Committee noted that grade 3-5 AEs associated with ASCT (ie reported in Group A only) included febrile neutropenia (20%), thrombocytopenia (49%), mucositis (9%), diarrhoea (5%) and infections (17%) at rates that members considered were considerably lower than what would be expected with standard of care ASCT in New Zealand. The Committee noted that participants in Group I completely avoided these transplant-related toxicities by not having an ASCT.

11.23.3. Maintenance phase: The Committee noted that half of all trial participants (balanced across groups) received rituximab maintenance, whilst all

participants in Group I received ibrutinib maintenance for two years. The Committee noted the following grade 3-5 AEs were increased with ibrutinib: neutropenia (23% Group I vs 17% Group A), infections (19% vs 13%) and cardiac events (4% vs 1%) and considered that these differences reflected a non-significant increase in events.

11.23.4. Members noted that cardiac events are of particular interest with ibrutinib and considered that the lower rate of cardiac events in TRIANGLE compared with the higher rate of cardiac toxicity and deaths reported in SHINE (which included a more elderly population who received ibrutinib until progression) likely reflects these differences in the patient cohorts and duration of ibrutinib treatment.

11.23.4.1. The Committee noted that the number of deaths reported in Groups A and I were similar across phases except for the ASCT phase (induction: 3 vs 2; ASCT: 9; maintenance 5 vs 3, the latter of which included one death due to myocardial infarction). The Committee noted a small number of secondary cancers were reported across groups but considered the true incidence unlikely to be identified by the 33-month follow up.

11.24. The Committee noted that TRIANGLE study had an open-label design, included complex statistical comparisons, and did not use a placebo control during the maintenance phase (which may have reduced bias and better managed participant dropout and changes between treatments). Members noted that 42 patients in Group A did not receive ASCT for various reasons including 'unknown' and clinician decision.

11.25. The Committee noted the other evidence from the application and identified by an additional literature search.

11.26. The Committee noted there was no patient preference or quality of life (QOL) data identified. The Committee considered it likely that people with MCL would prefer ibrutinib over ASCT given the treatment burden and risk profile of the latter and noting individuals would already be receiving maintenance treatment with rituximab for two years.

11.27. The Committee considered TRIANGLE was generalisable to the New Zealand MCL population as the concurrent treatments and disease severity of the cohort were similar. Overall, the Committee considered that TRIANGLE provided high quality evidence of numerically superior FFS and PFS with ibrutinib (although the superiorities were not established in statistically significant terms); clinically meaningful reductions in the risk of infection, mortality and other toxicities (associated with ASCT); additional substantial benefits from the avoidance of ASCT requirements and impacts for patients, family, whanau and wider society; and improved outcomes for people with high-risk MCL (eg p53mut).

BTKi for 1L MCL

11.28. The Committee was made aware of evidence for ibrutinib in the first-line transplant ineligible MCL population from the SHINE trial (Wang et al. N Engl J Med. 2022;386:2482-64), which reported a substantial improvement in median PFS without an OS benefit. Members noted that ibrutinib was used until progression which differs from its use in transplant-eligible disease.

11.29. Members considered that, while not reviewed at this meeting, the evidence from the ECOG ACRIN EA4181 (acalabrutinib) and forthcoming MANGROVE (zanubrutinib)

trials suggests a likely class effect among BTKi for PFS for first-line treatment of transplant-eligible MCL.

BTKi for relapsed or refractory (R/R) MCL (ranked proposals)

11.30. The Committee considered that if ibrutinib were funded for first-line treatment of MCL, subsequent treatment with a BTKi (eg ibrutinib or zanubrutinib) would be clinically appropriate for late-occurring relapse only (ie did not progress while on therapy). The Committee considered it would be reasonable to retry a BTKi (eg ibrutinib) to strengthen second-line therapy because chemotherapy is known to provide poor outcomes for relapsed MCL, especially following intensive therapy.

11.31. The Committee considered that first-line ibrutinib would be time-limited therapy and not expected to lead to development of refractory disease, however, members noted that BTK mutations can occur and confer resistance to all BTKi.

11.32. The Committee considered that no future clinical trials would be expected to investigate BTKi for relapsed or refractory MCL given that trials are now investigating third-generation BTKi's such as pirtobrutinib, BTK protein degraders, and cellular therapies eg CAR-T.

11.33. The Committee noted that, increasingly over the past few years, the international treatment paradigm for MCL has evolved to place BTKi first-line, followed by new therapies in subsequent lines. However, members considered that where chemotherapy was used first-line followed by BTKi until PD, this meant that upon progression, the disease would generally be refractory to BTKi and may have about 30% sensitivity to BTKi if trialled again (exceptions being those who discontinued due to toxicity and may retain sensitivity).

Suitability

11.34. The Committee considered that first-line ibrutinib for transplant-eligible MCL would offer substantial suitability benefits as it would change the treatment setting from an inpatient stay to an entirely outpatient regimen. The Committee considered that ibrutinib's medicine interactions (eg with warfarin) are manageable.

11.35. The Committee considered that there would be inequities in access for provincial or rural people with MCL that would be removed by funding ibrutinib first-line for MCL, due to it avoiding the need for ASCT and being used in the community.

11.36. The Committee considered that people living in rural or regional areas would experience substantial impacts from ASCT (eg from infection risk and inpatient stays associated with undergoing ASCT) and that less intensive therapy with ibrutinib, as an oral treatment, would offer considerable suitability benefits compared with ASCT.

Cost and savings

11.37. The Committee considered that the estimate of 29 patients per year was reasonable, noting this was based on cancer registry data and Australasian evidence for proportions receiving first-line treatment and being ASCT-eligible (Minson et al. Br J Haematol. 2024;204:548-54).

11.38. The Committee considered that avoiding the need for 29 ASCT per year would also provide an opportunity gain for the health system and other people by allowing other patients on waitlists (eg those with myeloma) to receive transplants sooner.

11.39. The Committee considered that the first-line chemotherapy regimen used with ibrutinib maintenance would be R-CHOP/RDHAP (DHAOx), following the prescriptive TRIANGLE protocol which has known safety. However, members considered that some clinicians might be comfortable omitting R-CHOP in favour of bendamustine

and rituximab (BR) by extrapolating evidence of superiority with this regimen from the STiL, BRIGHT, ENRICH or GALLIUM trials, or may omit RDHAP in favour of rituximab and cytarabine (RC) by extrapolating evidence of good outcomes from the Nordic MCL2 or Merryman trials. However, members also considered that there was some long-term uncertainty regarding the potential for relapse following maintenance due to a lack of very long-term data.

11.40. The Committee considered that the total cost of ASCT would need to include apheresis costs, bed costs, and the high cost of plerixafor.

11.41. The Committee considered that avoidance of ASCT and its associated requirements and impacts over the first 30 to 60 days, and the reduction in toxicity from ASCT would lead to reductions in healthcare resource use.

11.42. The Committee considered Pharmac staff's health resource utilisation (HRU) estimates for the ASCT phase (100 days post-transplantation) and maintenance phase (after 100 days post-transplantation). The Committee noted that the hospital readmission rate is about 25% in the first three months and considered that follow-up after three months would be expected to be similar in both groups because patients would be receiving rituximab maintenance eight-weekly in both situations. In the maintenance phase, members considered there would be fewer than 12 outpatient visits although this may be increased where needed to appropriately manage patients with high risk of cardiac events, or experiencing other AEs arising with ibrutinib maintenance.

11.43. The Committee considered that rituximab maintenance would be used for three years in patients who receive ibrutinib maintenance, aligned with the key clinical trial evidence.

11.44. The Committee considered that subsequent ASCT would be highly unlikely as patients would not likely be transplant eligible at the second line. Members considered this would be a very uncommon scenario as it is more challenging to harvest after chemotherapy, and disease would be refractory to chemotherapy by that time.

Funding criteria

11.45. The Committee considered that it would be straightforward to identify the target group for first-line treatment using funding criteria specifying transplant-eligible MCL.

11.46. The Committee considered it reasonable to specify that the chemotherapy regimen to be used with ibrutinib maintenance should be RCHOP/RDHAP (DHAOx), following the TRIANGLE protocol.

11.47. The Committee noted that patients need to be fit enough to receive high dose cytarabine as part of induction chemotherapy which may limit access to ibrutinib from beyond the intended group.

Summary for assessment

11.48. The Committee considered that the below summarises its interpretation of the most appropriate PICO (population, intervention, comparator, outcomes) information for ibrutinib if it were to be funded in New Zealand for first-line treatment of transplant-eligible MCL. This PICO captures key clinical aspects of the proposal and may be used to frame any future economic assessment by Pharmac staff. This PICO is based on the Committee's assessment at this time and may differ from that requested by the applicant. The PICO may change based on new information, additional clinical advice, or further analysis by Pharmac staff.

Population	Mantle cell lymphoma (MCL) in patients eligible for autologous stem cell transplant (ASCT) requiring first-line (1L) therapy.
Intervention	560mg daily days 1-19 of each 21-day RCHOP[#] chemotherapy cycle for a total of 3 cycles of induction chemotherapy, followed by fixed-duration maintenance ibrutinib (560 mg orally each day) for 2 years Also includes the following: • induction chemotherapy[#] • rituximab maintenance for 3 years* [#]The TRIANGLE protocol would be followed and one of the following chemotherapy regimens would be used: RCHOP/RDHAC/RHAOx/or another platinum-containing regimen. *Rituximab maintenance in TRIANGLE was 2-monthly for 3 years. Currently funding is only for 2 years of rituximab maintenance. Funding rituximab with ibrutinib (instead of ASCT for MCL) would be for 3 years of rituximab maintenance to follow the TRIANGLE protocol.
Comparator(s)	ASCT Also include the followings: • induction chemotherapy^ • rituximab maintenance for 2 years ^Induction chemotherapy varies and includes: Nordic protocol R-Maxi-CHOP x 3 and 3x R-high dose cytarabine, or Merrryman protocol R Bendamustine x 3 with R Cytarabine x 3 (RC.RB), or RDHAP or RDHOx.
Outcome(s)	Based on the evidence from the Triangle study, failure-free survival and progression-free survival are numerically superior but not statistically significant terms with ibrutinib (per TRIANGLE) vs ASCT. OS data immature For the duration of transplant and post-transplant phase (100 days post-transplantation) • Health resources utilisation markedly improved with ibrutinib vs ASCT • Health related quality of life markedly improved with ibrutinib vs ASCT
Table definitions: Population, the target population for the pharmaceutical; Intervention, details of the intervention pharmaceutical; Comparator, details the therapy(s) that the patient population would receive currently (status quo – including best supportive care); Outcomes, details the key therapeutic outcome(s) and source of outcome data.	

12. Blinatumomab for B cell lineage acute lymphoblastic leukaemia (ALL), measurable residual disease (MRD)-negative

Application

12.1. The Committee reviewed the application for blinatumomab for the treatment of B cell lineage acute lymphoblastic leukaemia (B-ALL), measurable residual disease (MRD)-negative.

12.2. The Committee took into account, where applicable, Pharmac's relevant decision-making framework when considering this agenda item.

Recommendation

12.3. The Committee **recommended** that blinatumomab for the treatment of MRD-negative B-cell lineage acute lymphoblastic leukaemia be funded with a **high priority** within the context of treatment of malignancy subject to the following Special Authority criteria:

> **Initial application –** B-cell acute lymphoblastic leukaemia/lymphoma in complete remission with negative minimal residual disease
>
> Applications from any relevant practitioner. Approvals valid for 3 months for patients meeting the following criteria:
> All of the following:
> 1. Patient has B-cell acute lymphoblastic leukaemia/lymphoma (ALL); and
> 2. Patient has received intensive combination chemotherapy for initial treatment of ALL or for subsequent salvage therapy; and
> 3. Patient has not previously received treatment with blinatumomab; and
> 4. Patient has received complete remission; and
> 5. Individual is minimal residual disease (MRD) negative, defined as 0.01% or fewer leukemic cells based on measurement in bone marrow, documented after an interval of at least 2 weeks from last systemic chemotherapy, measured using polymerase chain reaction or flow cytometry; and
> 6. Treatment is to be administered for a maximum of 4 cycles.
>
> **Renewal -** B-cell acute lymphoblastic leukaemia/lymphoma in complete remission with negative minimal residual disease
> Applications from any relevant practitioner. Approvals valid for 3 months for patients meeting the following criteria:
> Both:
> 1. Disease continues to be in complete remission; and
> 2. Treatment is to be administered for a maximum of 4 cycles.

12.4. The Committee considered the following in making their recommendation:

- the high unmet health need
- blinatumomab consolidation treatment is likely to decrease the number of individuals proceeding to an allogenic stem cell transplants in their first complete remission
- the increased chance of a cure for individuals
- the potential impact on future health resource and impact on family and whānau if individuals are either cured or do not proceed to allogenic stem cell transplantation.

Discussion

Māori impact

12.5. The Committee discussed the impact of funding blinatumomab for the treatment of MRD-negative B- ALL on Māori health areas of focus | Hauora Arotahi and Māori health outcomes.

12.6. The Committee previously noted an Auckland-based clinical audit of 65 patients, which reported that outcomes for Māori adults B-ALL were worse than for non-Māori (Hong Chun Wong et al. N Z Med J. 2023;136:10-18).

12.7. The Committee noted the study reported that the 3-year overall survival was 36 months for Māori/Pacific peoples versus 57 months for non-Māori and Pacific peoples and the event free survival being 21% for Māori/Pacific peoples in comparison to non-

Māori and Pacific peoples being 54% at 3 years (Hong Chun Wong et al. 2023). The Committee considered the study was a small retrospective study.

Populations with high health needs

12.8. The Committee discussed the health need(s) of individuals with MRD-negative B-ALL among Māori, Pacific peoples, disabled peoples including tāngata whaikaha Māori, and other populations identified by the Government Policy Statement on Health 2024-2027 to have high health needs. The Committee discussed the impact of funding blinatumomab and considered:

12.8.1. The Committee noted the three year overall survival results of Hong Chun Wong et al. 2023 reported that the three-year overall survival was 36 months for Māori/Pacific peoples versus 57 months for non-Māori and Pacific peoples. The Committee considered the study was a small retrospective study.

Background

12.9. The Committee noted that several therapies for the treatment of B-ALL have been reviewed and that these can be found on the application tracker.

12.10. The Committee noted that it previously considered blinatumomab for the treatment of MRD positive in B-ALL in October 2024 and recommended it for funding with a high priority.

12.11. The Committee considered treatment of MRD-negative ALL has not been previously considered.

Health need

12.12. The Committee considered the health need of individuals with B-ALL was reviewed by CTAC in October 2024.

12.13. The Committee considered MRD status is strongly and consistently associated with clinical outcomes in B-ALL. Adults with MRD-negative B-ALL after induction chemotherapy have a better prognosis than individuals with MRD-positive status. However, people with MRD-negative disease still frequently experience relapse, with event-free survival of 64% at 10 years compared with 21% for those who are MRD-positive (Berry et al. JAMA Oncol. 2017;3:e170580).

12.14. The Committee noted a meta-analysis of 39 studies reported that among paediatric patients, 77% of those who were MRD-negative were disease-free at 10 years compared with 32% of MRD-positive patients, and overall survival was 84% versus 55%, respectively. In adults, 64% of MRD-negative patients were disease-free at 10 years compared with 21% of MRD-positive patients, and overall survival was 60% versus 15% (Berry et al. JAMA Oncol. 2017;3:e170580).

12.15. The Committee noted Berry et al. 2017 and Shen et al. BMC Cancer 2018;18:755 that reported that individuals with MRD-positive disease prior to allogeneic stem cell transplantation (SCT) had a significantly higher rate of relapse compared with those with MRD-negative disease (HR = 3.26; P < 0.05). Pre-transplantation positive MRD was a significant negative predictor of relapse-free survival (RFS) (HR = 2.53; P < 0.05), event-free survival (EFS) (HR = 4.77; P < 0.05), and overall survival (OS) (HR = 1.98; P < 0.05).

12.16. The Committee considered in New Zealand it was possible to measure MRD status for individuals with Philadelphia negative B-ALL through flow cytometry, whilst for individuals with Philadelphia positive B-ALL this can be measured via both flow cytometry and polymerase chain reaction (PCR) assay, which is more sensitive. The Committee considered next generation sequencing has recently become available

and samples are sent away for testing in Australia. The Committee considered this was a small number of cases.

12.17. The Committee noted a small number of individuals per year had funded access to blinatumomab through the Named Patient Pharmaceutical Assessment pathway, these individuals had MRD positive disease following induction and consolidation chemotherapy as a bridge to a potentially curative allogeneic stem cell transplant.

12.18. The Committee considered most individuals would receive intensive chemotherapy, the Committee considered only those who were clinically frail may not receive this treatment.

12.19. The Committee considered individuals who experience disease relapse following consolidation therapy would receive inotuzumab as a bridge to allogenic stem cell transplant if being treated with a curative intent.

12.20. The Committee considered approximately 25% of people have Philadelphia positive B-ALL. The Committee considered approximately 75% with Philadelphia positive disease who have persistent MRD positivity or relapse have a kinase domain mutation which confers resistance to the currently funded treatment options. The Committee considered ponatinib has been reported to have efficacy for this group however is not funded via the schedule in New Zealand.

12.21. The Committee considered individuals aged 15-30 would be treated with a paediatric intensive protocol and considered to have a low risk of proceeding to allogenic stem cell transplant. Whilst those aged 31 – 60 are treated with a standard adult protocol with the main aim of consolidating with an allogenic transplant.

Health benefit

12.22. The Committee noted Litzow et al. N Engl J Med. 2024;391:320–33 that reported the results of the third interim analysis (medium follow up 43 months) of the E1910 trial.

12.22.1. The Committee noted that the overall survival at 3 years was 85% in the blinatumomab arm compared to 68% in the chemotherapy arm, hazard ratio for death 0.41 (95%CI: 0.23-0.73); P=0.002.

12.22.2. The Committee noted the relapse free survival at 3 years was 80% in the blinatumomab arm compared to 64% in the chemotherapy arm, hazard ratio for relapse or death was 0.53 (95%CI: 0.32-0.87).

12.22.3. The Committee noted that all individuals included in the trial were Philadelphia negative and aged between 30-70 (N=488).

12.22.4. The Committee noted the induction protocol treatments used in the trial were the same as those used in New Zealand.

12.22.5. The Committee noted the analysis focussed on the MRD negative group and considered that changes in worldwide funding of blinatumomab in the initial stages of the trial made it unethical to include those who had MRD positive disease.

12.22.6. The Committee considered all subgroups in the trial benefited from the addition of blinatumomab, however noted the trial was not powered for subgroup analysis.

12.22.7. The Committee noted there was an increase in grade three neurological or psychiatric events when blinatumomab was added to the consolidation chemotherapy, however there were no grade four or five events.

12.22.8. The Committee considered the majority of deaths in the trial were from disease relapse.

12.22.9. The Committee considered the trial was well designed and provided high quality evidence.

12.22.10. The Committee noted approximately 60% of the trial population had adverse risk B-ALL. The Committee considered that in New Zealand these individuals would be intended to undergo allogeneic stem cell transplant, however the trial protocol suggested only 30% in each group would proceed to transplant.

12.22.10.1. The Committee considered less than 30% in each arm did undergo an allogeneic stem cell transplant. The Committee considered the numbers in each arm were less than they would expect in the New Zealand setting. The Committee considered this may be due to individuals in the trial having better access to salvage options, or better access to more sensitive MRD testing.

12.23. The Committee considered the potential health benefit of blinatumomab for individuals aged between 15-30 who are treated on a paediatric inspirated, or paediatric protocol.

12.23.1. The Committee noted the COG AALL1731, a phase III study that included children ≤10 years of age, with standard risk Philadelphia negative B-ALL (Gupta et al N Engl J Med 2025;392:875-91). The Committee considered the addition of blinatumomab to consolidation chemotherapy resulted in a disease-free survival and overall survival benefit.

12.23.2. The Committee considered there was strong evidence of health benefit in individuals whose ages ranged either side of the 15-30 age group.

12.23.3. The Committee considered that the addition of blinatumomab to consolidation chemotherapy has become standard of care for adolescent and young adult patients being treated on either a paediatric inspirated, or paediatric protocol worldwide.

12.23.4. The Committee considered that for adolescent and young adult patients in New Zealand are unable to access new clinical trials where blinatumomab is included in the control arm as this is not currently funded for these individuals.

12.23.5. The Committee considered no new evidence would be generated in this group as it would not be ethical. The Committee considered new trials were designed to evaluate if blinatumomab was efficacious as a single agent without consolidative chemotherapy.

12.23.6. The Committee considered it was reasonable to assume that this group would receive clinical benefit from blinatumomab treatment.

12.24. The Committee considered only a small proportion of individuals over the age of 70 would be clinically fit enough for treatment with intensive chemotherapy.

12.25. The Committee considered the potential health benefit of blinatumomab for individuals with Philadelphia positive B-ALL in a MRD negative remission state.

12.25.1. The Committee considered there was a lack of evidence to evaluate the health benefit of blinatumomab treatment in individuals with Philadelphia positive B-ALL in a MRD negative remission state.

12.25.2. The Committee noted a small group of individuals (14%) with a Philadelphia like ALL were included in the E1910 trial. The Committee considered these

individuals did show health benefit from blinatumomab. The Committee considered that whilst the data could not be extrapolated to individuals with Philadelphia positive B-ALL in a MRD negative remission state, it would be reasonable to assume there would be efficacy in this group.

12.25.3. The Committee noted other international health technology agencies have assumed similar efficacy in individuals with Philadelphia positive disease. The Committee considered the agencies that have not assumed similar efficacy fund ponatinib for this group and therefore the unmet health need of these populations differ to that in New Zealand.

12.25.4. The Committee considered no new evidence would be generated in this group to determine if blinatumomab was efficacious. The Committee considered that new trials were designed to evaluate if upfront blinatumomab and tyrosine kinase inhibitor treatment was efficacious in these individuals without the addition of chemotherapy. The Committee noted this was a small phase II trial but had early promising results.

12.26. The Committee considered individuals with co-morbidities who are borderline for allogenic stem cell transplants would experience a high health benefit from blinatumomab treatment. The Committee considered these individuals would experience a reduction in transplant related mortality.

12.27. Overall, the Committee considered that the addition of blinatumomab to consolidation chemotherapy in adult patients in an MRD-negative remission from Philadelphia negative B-ALL significantly improved overall survival, increased the chance of a cure, and may decrease the likelihood of proceeding to allogeneic stem cell transplant.

12.28. The Committee considered it was reasonable to assume efficacy in individuals with Philadelphia positive B-ALL in a MRD negative remission state and in those who are aged 15-30.

12.29. The Committee also considered blinatumomab treatment for individuals with MRD positive disease, may enable them to transition to an MRD negative disease state to optimise the benefit of their consolidative allogeneic stem cell transplant.

Suitability

12.30. The Committee considered that blinatumomab may reduce the need for an allogenic stem cell transplant which would have a large impact on an individual's ability work, and time away from whānau.

12.31. The Committee considered that blinatumomab would mostly be administered as an outpatient regimen. The Committee considered there was no limitations on the supply of Cassette pumps currently in New Zealand. The Committee considered blinatumomab would not be a burden to the individual compared to currently funded options.

Cost and savings

12.32. The Committee considered that approximately 10 patients per year would have MRD negative disease following induction and consolidation and therefore be eligible for blinatumomab treatment in New Zealand.

12.33. The Committee considered approximately 18-20 individuals each year are ≥15 years with newly diagnosed B-ALL are treated with an intensive protocol with curative intent in New Zealand. Approximately 20-25% have Philadelphia positive B-ALL.

12.34. The Committee considered that access to blinatumomab would reduce the number of individuals in New Zealand in an MRD-negative remission from Philadelphia negative B-ALL undergoing an allogeneic stem cell transplant.

12.35. The Committee considered individuals with high-risk genomic lesions who are MRD negative after induction are unlikely to directly proceed to allogenic stem cell transplant in New Zealand. The Committee considered the average wait time for the transplant is 9-12 months. The Committee considered these individuals would access blinatumomab if funded while waiting for the transplant.

12.36. The Committee considered individuals who are MRD positive after induction would access blinatumomab to optimise their benefit from allogenic stem cell transplants. The Committee considered it was unlikely that these individuals would progress to a non-transplant pathway following their first complete remission.

12.37. The Committee noted the majority of individuals in the E1910 trial had the full four cycles of blinatumomab. The Committee considered if blinatumomab were funded most would receive four cycles due to the lack of timely access to allogenic stem cell transplants. The Committee noted Luger et al Blood 2023;142: 2877., that reported the results of a small, unplanned subgroup analysis that suggested that individuals that received four cycles of blinatumomab had the most significant overall survival benefits. The Committee considered there were multiple confounding factors in the study and no clear conclusion could be drawn without additional evidence.

12.38. The Committee considered that MRD positive disease would also receive four cycles of blinatumomab.

12.39. The Committee considered it would be reasonable to assume that no patients would receive an allogenic stem cell transplant before completing consolidation treatment in New Zealand. The Committee considered current long wait times were not clinically appropriate, however, were based on health system resource factors.

12.40. The Committee considered it was reasonable to extrapolate the survival benefit beyond the trial period, the survival curve remained stable for at two years.

12.41. The Committee considered it was reasonable to assume that individuals whose disease remains relapse free after five years are cured.

Funding criteria

12.42. The Committee considered individuals who experienced disease relapse whilst being treated with blinatumomab should not receive blinatumomab treatment in subsequent lines of therapy.

Summary for assessment

12.43. The Committee considered that the below summarises its interpretation of the most appropriate PICO (population, intervention, comparator, outcomes) information for blinatumomab if it were to be funded in New Zealand for MRD negative B-ALL. This PICO captures key clinical aspects of the proposal and may be used to frame any future economic assessment by Pharmac staff. This PICO is based on the Committee's assessment at this time and may differ from that requested by the applicant. The PICO may change based on new information, additional clinical advice, or further analysis by Pharmac staff.

Population	People with MRD-negative B-cell lineage acute lymphoblastic leukaemia (ALL)

Intervention	Blinatumomab plus chemotherapy as part of consolidation treatment, likely in the following sequence based on the E1910 trial (Litzow et al. N Engl J Med. 2024;391(4):320–333)): • 2 cycles of blinatumomab • 3 cycles of chemotherapy • 1 cycle of blinatumomab • 1 cycle of chemotherapy • 1 cycle of blinatumomab Blinatumomab is administered by continuous intravenous infusion of 28 mcg blinatumomab per day for 28 days followed by a 14-day treatment free period.
Comparator(s)	Four cycles of chemotherapy alone as part of consolidation treatment, based on the E1910 trial (Litzow et al. 2024) It is acknowledged that some patients may receive alternative induction regimes, such as Hyper-CVAD and UKALL14 protocols (Marks et al. Lancet Haematol. 2022; 9: e262-e27, Kantarjian et al, Cancer. 2004;101:2788-801).
Outcome(s)	Improved OS compared with chemotherapy alone (85% vs. 68% at three years; hazard ratio for death: 0.41) (Litzow et al. 20243) Improved RFS compared with chemotherapy alone (80% vs. 64% at three years; hazard ratio for relapse or death: 0.53) (Litzow et al. 2024).
Table definitions: Population, the target population for the pharmaceutical; Intervention, details of the intervention pharmaceutical; Comparator, details the therapy(s) that the patient population would receive currently (status quo – including best supportive care); Outcomes, details the key therapeutic outcome(s) and source of outcome data.	

13. Matters Arising - Rule 8.1b AYA access options – follow-up discussion

Recommendation

13.1. The Committee supported the proposed three-part eligibility model as a clinically appropriate and workable basis for determining access under Rule 8.1b for adolescents and young adults with paediatric-origin cancers.

13.2. The Committee supported the use of tumour biology and treatment on a paediatric-based protocol, rather than age alone, as the basis for determining eligibility.

13.3. The Committee supported the proposed list of paediatric-origin cancers, noting that it should be subject to ongoing clinical review.

Discussion

13.4. The Committee noted an update from Pharmac staff on a refined approach to expanding access under Rule 8.1b for adolescents and young adults (AYA) with paediatric-origin cancers, incorporating feedback from the Committee's November 2025 discussion and subsequent consideration by the Pharmac Board.

13.5. The Committee noted the proposed three-part eligibility model requiring:

i. a paediatric-origin cancer
ii. treatment on a paediatric-based protocol, and
iii. eligibility for AYA cancer services under the Te Whatu Ora Tier 3 Service Specification.

13.6. The Committee considered that the proposed model appropriately aligns access with tumour biology and treatment protocol, rather than chronological age alone.

13.7. The Committee supported the proposed eligibility framework as clinically appropriate and a practical basis for determining access under Rule 8.1b for AYA.

13.8. The Committee considered that the requirement for treatment on a paediatric-based protocol provides an important mechanism to ensure access is limited to circumstances where paediatric-based care is clinically indicated.

13.9. The Committee supported the use of AYA service eligibility criteria as a flexible and established mechanism for determining access, including continuity of care where patients move beyond the typical age range during treatment.

13.10. The Committee noted that the combined application of all three criteria appropriately constrains access and does not represent a broad extension based on age alone.

13.11. The Committee considered that defining a list of paediatric-origin cancers provides useful clinical boundaries to support consistent application across paediatric and adult services and supported the proposed list as a reasonable basis for operationalising the model.

13.12. The Committee discussed acute lymphoblastic leukaemia (ALL), noting its occurrence across wider age groups, and considered that the proposed model appropriately manages potential scope through the requirement for a paediatric-based protocol and AYA service involvement.

13.13. The Committee considered that multidisciplinary team (MDT/MDM) oversight would be important to ensure consistent and clinically appropriate application of the criteria in practice.

13.14. The Committee noted that the illustrative scenarios provided were helpful in demonstrating how the proposed approach would operate in practice.

Stephen Munn _____ _____

Chair Date