Appendix One - Update on medicine applications involved in the petitions to Parliament

PHARMAC's funding process

PHARMAC has an established process for deciding which medicines to fund. The following information provides an overview of the key stages in PHARMAC's processes for assessing funding applications for new medicines or for wider access to already funded medicines.

Stages in PHARMAC's funding process	
1. Application	Applicant (pharmaceutical supplier, clinician, consumer) submits a funding application to PHARMAC.
2. Initial assessment	PHARMAC reviews the funding application, collates additional information and undertakes preliminary analysis.
3. Expert advice	PHARMAC seeks advice from expert clinical advisors, who make funding priority recommendations to PHARMAC (see table below).
4. PHARMAC assessment	PHARMAC assesses the funding application using the Factors for Consideration framework.
5. Ranked	PHARMAC compares and ranks the new funding application against other open medicine funding options. This process occurs at least four times per year. The ranking of all medicines already ranked is also reviewed each time.
6. Negotiation	Once sufficient funding is available to potentially fund the next medicine on the ranking list, PHARMAC negotiates a provisional contract with the supplier.
7. Consultation	PHARMAC consults with suppliers, healthcare professionals and consumers and the general public on a proposal to fund (or decline funding for) the medicine.
8. Decision	PHARMAC considers all feedback from the consultation and makes a decision on funding.
9. Notification	PHARMAC notifies the decision and activates implementation support as required.

PHARMAC may, at its discretion, adopt a different process or variations of this process. For instance, funding applications may be reassessed by PHARMAC's expert clinical advisors a number of times, due to new evidence and/or the need to seek specialist advice from a range of different experts.

Table 1: Update on funding applications from the petitions submitted to Parliament as at 11 October 2019

Medicine	Indication	Application received	Clinical recommendation	Stage	Comments	Updates since May 2019
Pembrolizumab (Keytruda) PHARMAC has received three funding applications for pembrolizumab (Keytruda) for lung cancer.	Non-small cell lung cancer, locally advanced or metastatic, PD-L1 positive, second-line	September 2016	Low	5. Ranked	• This funding application was assessed by PTAC twice, in November 2016 and August 2017. It was also assessed by CaTSoP in March 2017. • First ranked by PHARMAC in November 2016. • See https://pharmac.govt.nz/wwwtrs/ApplicationTracker.php?ProposalId=1467	PHARMAC plans to run a competitive process for the supply of immune checkpoint inhibitors for lung cancer, and possibly other new indications. We are aiming to release this in late 2019 or early 2020.
	Non-small cell lung cancer, PDL1 positive, first-line	February 2017	Medium	5. Ranked	• This funding application was assessed by PTAC in May 2017, November 2017, August 2018, November 2018, February 2019 and CaTSoP twice, in March 2017 and April 2019 (CaTSoP April 2019 advice & recommendations pending publication). • First ranked by PHARMAC in March 2019. • See https://pharmac.govt.nz/wwwtrs/ApplicationTracker.php?ProposalId=1510	
	Non-small cell lung cancer, metastatic, in combination with chemotherapy, first-line	August 2018	Medium	5. Ranked	• This funding application was assessed by PTAC twice, in November 2018 and February 2019. It was also assessed by CaTSoP in April 2019. • First ranked by PHARMAC in March 2019. • See https://pharmac.govt.nz/wwwtrs/ApplicationTracker.php?ProposalId=1690	
Alectinib (Alecensa)	Non-small cell lung cancer, advanced, anaplastic lymphoma kinase-positive, first-line	May 2018	Medium High	9. Notification	• This funding application was assessed by PTAC in August 2018. PTAC referred the application to CaTSoP for advice regarding appropriate Special Authority criteria. CaTSoP assessed this application again in April 2019. • First ranked by PHARMAC in September 2018. • See https://pharmac.govt.nz/wwwtrs/ApplicationTracker.php?ProposalId=1670	PHARMAC has consulted on and notified its decision to fund alectinib for non-small cell lung cancer from 1 December 2019
Osimertinib (Tagrisso)	Non-small cell lung cancer, locally advanced or metastatic, EGFR T790M positive	November 2017	Defer	3. Expert advice	• CaTSoP assessed this in April 2018 and September 2018 and deferred making a recommendation, pending data from a study that is underway. • See https://pharmac.govt.nz/wwwtrs/ApplicationTracker.php?ProposalId=1593	
Crizotinib (Xalkori)	Non-small cell lung cancer, advanced, anaplastic lymphoma kinase-positive	August 2015	Decline Medium	5. Ranked	• This funding application was assessed by PTAC in November 2015, August 2016, and August 2018. It was also assessed by CaTSoP in May 2016 and April 2019. (CaTSoP recommended funding at medium priority). • Our clinical advisors originally recommended crizotinib be declined due to concerns around the trial design, a lack of long-term data and poor cost effectiveness given the price being sought from the supplier. • First ranked by PHARMAC in November 2016. • See https://pharmac.govt.nz/wwwtrs/ApplicationTracker.php?ProposalId=1373	Now "under assessment", but any reranking would be in the context of a funding decision on alectinib (see above), as they would treat the same patient population.
Lenalidomide Lenalidomide was funded in 2014 for people with relapsed refractory multiple myeloma, subject to criteria.	Multiple myeloma, maintenance following frontline autologous SCT	November 2017	Medium	5. Ranked	• This funding application was assessed by CaTSoP in April 2018. • First ranked by PHARMAC in September 2018. • See https://pharmac.govt.nz/wwwtrs/ApplicationTracker.php?ProposalId=1590	
	Multiple myeloma, first-line treatment, ASCT-eligible	May 2017	Defer	3. Expert advice	• This funding application was assessed by CaTSoP in April 2018. • Our clinical advisors have recommended the application be deferred until there is more clinical evidence of health benefits. • See https://pharmac.govt.nz/wwwtrs/ApplicationTracker.php?ProposalId=1687	
	Multiple myeloma, first line, grade 3 or greater pre-existing neuropathy	April 2016	Medium High	4. PHARMAC assessment	• This funding application was assessed by CaTSoP in April 2016 and PTAC in August 2016. • Our clinical advisors recommended funding with medium/high priority and noted that patient numbers in this group were very small. • To date, PHARMAC has assessed funding for these patients via its Named Patient Pharmaceutical Assessment (NPPA) pathway. • See https://pharmac.govt.nz/wwwtrs/ApplicationTracker.php?ProposalId=1466	

Medicine	Indication	Application received	Clinical recommendation	Stage	Comments	Updates since May 2019
PHARMAC has received four funding applications to widen access to this treatment.	Multiple myeloma - first-line treatment - ASCT ineligible	May 2016	Cost-neutral	5. Ranked	• This funding application was assessed by PTAC in August 2017 and CaTSoP in April 2018. • Our clinical advisors recommended lenalidomide only be funded if cost-neutral to the health sector, compared an already funded treatment which provides the same or similar benefits. • First ranked by PHARMAC in December 2018 • https://pharmac.govt.nz/wwwtrs/ApplicationTracker.php?ProposalId=1462 • PHARMAC has not at this time received a cost-neutral proposal from a supplier of lenalidomide.	
Olaparib (Lynparza)	Ovarian cancer, relapsed, BRCA-mutated, platinum sensitive	February 2017	Medium	7. Consultation	• This funding application was assessed by CaTSoP in April 2018 and PTAC in November 2018. CaTSoP assessed the application in April 2019. • First ranked by PHARMAC in September 2019. • See https://pharmac.govt.nz/wwwtrs/ApplicationTracker.php?ProposalId=1511	On 1 September 2019 PHARMAC consulted to fund olaparib for BRCA-mutated relapsed ovarian cancer from 1 February 2020. A decision will be made by the Board when it meets in late November 2019.
Bevacizumab (Avastin)	Ovarian cancer, advanced	November 2013	Decline	5. Ranked	• This funding application was assessed by PTAC twice, in February 2014 and February 2016. It was also assessed by CaTSoP twice, in October 2014 and September 2015. • Our clinical advisors recommended bevacizumab be declined. While the data was encouraging, there was limited evidence of efficacy, a high price being sought by the supplier and the unapproved dosing regimen proposed. • First ranked by PHARMAC in May 2014. • See https://pharmac.govt.nz/wwwtrs/ApplicationTracker.php?ProposalId=1174	
Ibrutinib PHARMAC has received three funding applications for Ibrutinib.	Relapsed or refractory chronic lymphocytic leukaemia and chronic lymphocytic leukaemia (CLL) with an unfavourable 17p deletion genetic mutation.	August 2015	Medium	5. Ranked	• This funding application was assessed by PTAC four times, in November 2015, August 2016, November 2016 and February 2017. It was also assessed by CaTSoP in May 2016 and September 2016. • Initially it was recommended for decline but, having reviewed more evidence, our clinical advisors eventually recommended this as a medium priority for funding for the 17p deletion group and a low priority for other groups. • First ranked by PHARMAC in November 2016 • See https://pharmac.govt.nz/wwwtrs/ApplicationTracker.php?ProposalId=1374	
			Low	5. Ranked		
	Relapsed/ or refractory mantle cell lymphoma (progressed within 24 months of allograft/chemotherapy/chemoimmunotherapy)	August 2015	Low	5. Ranked	• This funding application was assessed by PTAC twice, in November 2015 and August 2016. It was also assessed by CaTSoP in May 2016. • First ranked by PHARMAC in November 2016. • See https://pharmac.govt.nz/wwwtrs/ApplicationTracker.php?ProposalId=1398	
	CLL, first line in unfit patients and relapsed/refractory Waldenstrom macroglobulinemia.	March 2018	No formal recommendation made	3. Expert advice	• This funding application was assessed by CaTSoP in September 2018. • There is no formal recommendation at this stage, as our clinical advisors noted that patient numbers in this group were very small. They it a low priority for a full evaluation and it would be preferable to consider widening access, should ibrutinib be funded for other populations with a higher health need. • See https://pharmac.govt.nz/wwwtrs/ApplicationTracker.php?ProposalId=1665	
Daratumumab	Relapsed or refractory myeloma	November 2017	Defer	3. Expert advice	• This funding application was assessed by CaTSoP in April 2018 and by PTAC in February 2019. • Our clinical advisors recommended this application be deferred until there is more clinical evidence of benefits. • See https://pharmac.govt.nz/wwwtrs/ApplicationTracker.php?ProposalId=1606	
Carfilzomib	Relapsed or refractory multiple myeloma	August 2018	Low	4. PHARMAC assessment	• This funding application was assessed by PTAC in February 2019 and will be ranked • See https://pharmac.govt.nz/wwwtrs/ApplicationTracker.php?ProposalId=1700	

Medicine	Indication	Application received	Clinical recommendation	Stage	Comments	Updates since May 2019
Venetoclax PHARMAC has received three funding applications for venetoclax.	Relapsed or refractory CLL (in combination with rituximab)	November 2018	**High**	7. Consultation	• This funding application was recently submitted to PHARMAC. It has been referred to CaTSoP and was assessed in April 2019. • First ranked by PHARMAC in June 2019. • See https://pharmac.govt.nz/wwwtrs/ApplicationTracker.php?ProposalId=1720	On 1 September 2019 PHARMAC consulted to fund venetoclax for chronic lymphocytic leukaemia from 1 February 2020. A decision will be made by the Board when it meets in late October 2019.
	Relapsed or refractory CLL with 17p deletion mutation	November 2017	**High**	7. Consultation	• This funding application was assessed by PTAC in February 2018 and CaTSoP in September 2018. It was assessed by PTAC in February 2019. • First ranked by PHARMAC in June 2019. • See https://www.pharmac.govt.nz/wwwtrs/ApplicationTracker.php?ProposalId=1595	
	Relapsed or refractory CLL with no other suitable treatment options	November 2017	**High** **Medium**	7. Consultation 7. Consultation	• This funding application was assessed by PTAC in February 2018 and CaTSoP in September 2018. • Our clinical advisors recommended it for funding for two different groups (depending on the length of pre-treatment), one with a high priority and one with a medium priority. • See https://pharmac.govt.nz/wwwtrs/ApplicationTracker.php?ProposalId=1596	
Pomalidomide	Relapsed or refractory multiple myeloma	November 2015	**Low**	5. Ranked	• This funding application was assessed by PTAC twice, in February 2016 and August 2016. It was also assessed by CaTSoP in April 2016. • First ranked by PHARMAC in September 2017. • See https://pharmac.govt.nz/wwwtrs/ApplicationTracker.php?ProposalId=1391	
Elotuzumab	Multiple myeloma	NA			• PHARMAC has not received a funding application for elotuzumab.	
Ixazomib	Multiple Myeloma	NA			• PHARMAC has not received a funding application for ixazomib.	
Nusinersen (Spinraza)	Spinraza for spinal muscular atrophy	August 2018	**Defer**	3. Expert advice	• This funding application was considered by the Rare Disorders Subcommittee in November 2018 and PTAC in May 2019. • Our clinical advisors have deferred making a recommendation, pending more evidence from two long-term clinical trials. • PHARMAC is in regular contact with the supplier and expects to receive interim data by the end of 2019. • See https://pharmac.govt.nz/wwwtrs/ApplicationTracker.php?ProposalId=1694 and https://pharmac.govt.nz/wwwtrs/ApplicationTracker.php?ProposalId=1730	Additional data has been received from the supplier and was considered by the Rare Disorders Subcommittee (RDSC) on 24 September. A record of the Subcommittee's advice and recommendations will be published in due course. PTAC will consider the recommendations made by the RDSC at its February meeting. However, PHARMAC may progress further assessment of any of the funding proposals considered at the RDSC meeting on the basis of the Subcommittee's advice, ahead of PTAC's review of the minutes.
Alglucosidase alfa (Myozyme) Myozyme is funded for infantile Pompe disease.	Late onset Pompe disease	December 2008 (clinician) and May 2016 (supplier)	**Decline**	5. Ranked	• The December 2008 application was from a clinician and provided very limited information. • A detailed application was received from the supplier in May 2016. • This funding application has been assessed by PTAC four times, in February 2009, November 2011, November 2016 and February 2019. It was also assessed by the Rare Disorders Subcommittee in November 2018. • Our clinical advisors have consistently recommended this for decline due to uncertainties regarding survival benefit, modest clinical benefits and the high proposed cost of the medicine. • First ranked by PHARMAC in November 2013 • See https://pharmac.govt.nz/wwwtrs/ApplicationTracker.php?ProposalId=182 and https://pharmac.govt.nz/wwwtrs/ApplicationTracker.php?ProposalId=1491	

Table 2: Targeted and hormonal therapies included in ESMO guidelines and the funding status in New Zealand as at 11 October 2019

* Listed on the Pharmaceutical Schedule without restrictions.

† Special authority criteria (which restrict funded access to specified clinical circumstances) apply.

‡ Has not previously been considered by PHARMAC for the treatment of breast cancer.

Text in yellow indicates information updated since May 2019.

Therapy Type	Medicine	Registered for breast cancer in NZ	Currently funded by PHARMAC	Application received	Comments	Clinical priority	Stage
Chemotherapy	Oxaliplatin	✗	✓ *				Fully funded
	Cisplatin	✗	✓ *				Fully funded
	Carboplatin	✗	✓ *				Fully funded
	Docetaxel	✓	✓ *				Fully funded
	Paclitaxel	✓	✓ *				Fully funded
	Doxorubicin	✓	✓ *				Fully funded
	Epirubicin	✓	✓ *				Fully funded
	Vinorelbine	✓	✓ *				Fully funded
	Capecitabine	✓	✓ *				Fully funded
	Cyclophosphamide	✓	✓ *				Fully funded
	Gemcitabine	✓	✓ *				Fully funded
	Methotrexate	✓	✓ *				Fully funded
	Fluorouracil	✓	✓ *				Fully funded
	Mitomycin	✓	✓ *				Fully funded
	Mitozantrone	✓	✓ *				Fully funded

Therapy Type	Medicine	Registered for breast cancer in NZ	Currently funded by PHARMAC	Application received	Comments	Clinical priority	Stage
	Nab-paclitaxel	✗	✗	August 2010	• This funding application has been assessed by PTAC six times, in November 2010, February 2011, August 2013, February 2014, August 2014 and May 2019. • It was also assessed by CaTSoP three times, in November 2010, September 2013 and March 2014. • Our clinical advisors recommended this for funding only if cost neutral. • First ranked by PHARMAC in November 2010.	**Cost-neutral**	**5. Ranked**
	Liposomal doxorubicin	✗	✗		• PHARMAC has not received a funding application for use as a breast cancer treatment.		
Hormonal Therapy	Anastrozole (Rolin)	✓	✓ *				Fully funded
	Exemestane	✓	✓ *				Fully funded
	Letrozole (Letrole)	✓	✓ *				Fully funded
	Tamoxifen (Genox)	✓	✓ *				Fully funded
	Fulvestrant (Faslodex)	✗	✗	August 2006	• This funding application has been assessed by PTAC in November 2006, July 2008, and February 2019. It was also assessed by CaTSoP in March 2008 and September 2018. • First ranked by PHARMAC in November 2013. • On 1 September 2019 PHARMAC consulted on a proposal to fund fulvestrant as second-line treatment for locally advanced or metastatic oestrogen receptor positive breast cancer as soon as practicable following Medsafe approval. • See https://www.pharmac.govt.nz/wwwtrs/ApplicationTracker.php?ProposalId=69	**Medium**	**7. Consultation**
Targeted Therapy	Eribulin‡ (Halaven)	✗	✗	NA	• PHARMAC has not received a funding application.		
	Everolimus‡ (Afinitor)	✗	✗	July 2018	• The funding application for advanced breast cancer was assessed by PTAC in May 2019. • First ranked by PHARMAC in June 2019. • See https://connect.pharmac.govt.nz/apptracker/s/application-public/a0R2P000000LuEU/everolimus	**Decline**	**5. Ranked**
	Palbociclib (Ibrance)	✓	✗	February 2018	• The funding application for first-line endocrine therapy was assessed by CaTSoP in September 2018. • PTAC assessed this funding application in May 2019. • First ranked by PHARMAC in March 2019. • RFP issued on 1 September 2019 seeking bids for the supply of CDK4/CDK6 inhibitor (ribociclib/palbociclib) for breast cancer • See https://www.pharmac.govt.nz/wwwtrs/ApplicationTracker.php?ProposalId=1631	**Low** **Medium**	**5. Ranked**
		✓	✗	November 2018	• The funding application for second-line endocrine therapy was assessed by CaTSoP in April 2019. In February 2019 PTAC requested to assess the application alongside the first-line application. This assessment took place in May 2019. • First ranked by PHARMAC in June 2019. • RFP issued on 1 September 2019 seeking bids for the supply of CDK4/CDK6 inhibitor (ribociclib/palbociclib) for breast cancer • See https://www.pharmac.govt.nz/wwwtrs/ApplicationTracker.php?ProposalId=1724	**Medium**	**5. Ranked**
	Ribociclib‡ (Kisqali)	✗	✗	April 2019	• PTAC assessed this funding application in May 2019. • First ranked by PHARMAC in September 2019. • RFP issued on 1 September 2019 seeking bids for the supply of CDK4/CDK6 inhibitor (ribociclib/palbociclib) for breast cancer	**Low**	**5. Ranked**

Therapy Type	Medicine	Registered for breast cancer in NZ	Currently funded by PHARMAC	Application received	Comments	Clinical priority	Stage
	Lapatinib (Tykerb)	✓	✓ †				Funded for metastatic breast cancer, first-line
	Neratinib (Nerlynx)	✗	✗		• PHARMAC has not received a funding application.		
	Pertuzumab (Perjeta)	✓	✓ †	January 2017 and November 2017	• Our clinical advisors recommended this for decline for HER-2 positive locally advanced, inflammatory or early stage, neoadjuvant treatment. • Our clinical advisors recommended this for decline for previously treated (second-line) HER-2 positive metastatic breast cancer.	**Decline**	Funded for HER-2 positive metastatic breast cancer, treatment naïve (first-line).
	Trastuzumab (Herceptin)	✓	✓ †				Funded for HER-2 positive early and metastatic breast cancer.
	Trastuzumab emtansine (TDM-1) (Kadcyla)	✓	✗	August 2017	• This funding application was assessed by PTAC in November 2017 and February 2019, and CaTSoP in September 2018. • First ranked by PHARMAC in December 2017. • PTAC assessed the application in February 2019 and made no recommendation. CaTSoP recommended the application be funded with medium priority in September 2019. • PHARMAC notified decision to fund trastuzumab emtansine for breast cancer from 1 December 2019. • See https://www.pharmac.govt.nz/wwwtrs/ApplicationTracker.php?ProposalId=1571	**Medium**	**9. Notification**
	Bevacizumab (Avastin)	✓	✗		• PHARMAC has not received a funding application for the treatment of breast cancer.		
	Olaparib (Lynparza)	✗	✗		• PHARMAC has not received a funding application for the treatment of breast cancer.		
	Talazoparib	✗	✗		• PHARMAC has not received a funding application.		
Checkpoint Inhibitors‡	Pembrolizumab/ Nivolumab/ Atezolizumab/ Durvalumab/ Avelumab	✗	✗		• PHARMAC has not received a funding application for the treatment of breast cancer.		
	Ipilimumab (Yervoy)	✗	✗		• PHARMAC has not received a funding application for the treatment of breast cancer.		