PHARMAC
TE PĀTAKA WHAIORANGA

REPORT FOR QUARTER FOUR 2019/20
INCORPORATING MONTHLY REPORT FOR JULY 2020

COMMERCIAL IN CONFIDENCE

Date **6 August 2020**

Attention **Hon Mr Chris Hipkins**
Minister of Health

Copies to:

PHARMAC Board
Lead DHB Chief Executive, Pharmaceuticals
Director-General of Health – Ministry of Health
Manager Governance and Crown Entities – Ministry of Health
Principal Advisor, Crown Entity Monitoring and Appointments – Ministry of Health

Contact(s)

Sarah Fitt, Chief Executive s9(2)(a)
Alison Hill, Director Engagement and Implementation s9(2)(a)

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Elexacaftor/ tezacaftor/ ivacaftor and ivacaftor (Trikafta)

There has recently been publicity around the cystic fibrosis medicine Trikafta, a combination medicine containing elexacaftor, tezacaftor, ivacaftor(75mg) and ivacaftor(150mg). PHARMAC has not yet received a funding application from the pharmaceutical supplier, Vertex, and Trikafta is not approved by Medsafe. PHARMAC approved funding for another cystic fibrosis medicine, Ivacaftor (brand name Kalydeco) also supplied by Vertex in February this year. At this time PHARMAC advised Vertex that we would be pleased to receive and assess a funding application from them for Trikafta.

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PHARMAC
TE PĀTAKA WHAIORANGA

BRIEFING

Medicines for Rare Disorders

Date: **19 February 2021**

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

Copies to: Director General of Health
PHARMAC Board
Lead DHB Chief Executive, Pharmaceuticals
Principal Advisor, Governance and Crown Entities, Ministry of Health

Contact(s)

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

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Trikafta

We are aware of public interest in a new treatment for cystic fibrosis, Trikafta, which is a combination of three different medicines - elexacaftor, tezacaftor and ivacaftor.

Potentially around 500 people would benefit from this medicine, so it does not meet our definition of 'rare'. However, Rare Disorders New Zealand (and other advocacy groups for cystic fibrosis) consider medicines for cystic fibrosis in the same way as other rare disorders medicines.

While available in some countries, Trikafta is not registered in New Zealand or Australia. An application for registration hasn't been submitted to Medsafe and PHARMAC has not received a funding application for it. We have invited the supplier, Vertex, to apply for Medsafe registration and PHARMAC funding, however, we can't start our assessment of the evidence without detailed information from the supplier (including a price offer).

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PHARMAC
TE PĀTAKA WHAIORANGA

MONTHLY REPORT FOR FEBRUARY 2021

COMMERCIAL IN CONFIDENCE

Date **3 March 2021**

Attention **Hon Andrew Little**
Minister of Health

Copies to:

PHARMAC Board
Lead DHB CE, Pharmaceuticals
Director-General of Health – Ministry of Health
Manager Governance and Crown Entities – Ministry of Health
Principal Advisor, Crown Entity Monitoring and Appointments – Ministry of Health

Contact(s)

Sarah Fitt, Chief Executive s9(2)(a)
Alison Hill, Director Engagement and Implementation s9(2)(a)

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TV3 series on Trikafta

On 1-3 February 2021, TV3 aired a three-part series on the cystic fibrosis medicine, Trikafta, as a follow up to a similar TV3-produced series on Trikafta in 2020.

PHARMAC reiterated that we are aware of the keen interest in Trikafta and we have invited the supplier, Vertex, on a number of occasions to apply for Medsafe registration and submit a funding

application to PHARMAC. We cannot start our assessment of the evidence without detailed information from the supplier, including a price offer.

PHARMAC also highlighted to media and patient reports that Trikafta costs approximately $430,000 per year per person and that there are approximately 450 people in New Zealand who could benefit from this medicine. This means that (before any discounts PHARMAC might be able to negotiate) Trikafta could cost New Zealand approximately $200 million a year, which is around 20% of the current available budget for medicines).

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PHARMAC
TE PĀTAKA WHAIORANGA

REPORT FOR QUARTER 3 2020/21

INCORPORATING MONTHLY REPORT FOR APRIL 2021

COMMERCIAL IN CONFIDENCE

Date **5 May 2021**

Attention **Hon Andrew Little**
Minister of Health

Copies to:

PHARMAC Board
Lead DHB CE, Pharmaceuticals
Director-General of Health – Ministry of Health
Manager Governance and Crown Entities – Ministry of Health
Principal Advisor, Crown Entity Monitoring and Appointments – Ministry of Health

Contact(s)

Sarah Fitt, Chief Executive s9(2)(a)
Alison Hill, Director Engagement and Implementation s9(2)(a)

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Funding application for Trikafta

There is continued public and media interest in Trikafta, a medicine for people with cystic fibrosis. On 25 March the Australian regulator (the TGA) registered Trikafta. The Pharmaceutical Benefits Advisory Committee (PBAC) assessed Trikafta for funding at its March meeting. At this meeting, it deferred making a recommendation to list Trikafta for the treatment of people with cystic fibrosis, aged 12 years and older who have at least one F508del mutation in the CFTR gene.

PHARMAC staff attended a meeting on 30 March with representatives from the supplier (Vertex), Medsafe, and two consumer advocacy groups, Trikafta for Kiwis and Cystic Fibrosis NZ. We have been advised by Vertex that they are actively working to make an application to PHARMAC for funding. We have invited Vertex to apply for PHARMAC funding at the same time as Medsafe registration so that both organisations can consider Trikafta in parallel

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PHARMAC
TE PĀTAKA WHAIORANGA

MONTHLY REPORT FOR JUNE 2021

COMMERCIAL IN CONFIDENCE

Date **30 June 2021**

Attention **Hon Andrew Little**
Minister of Health

Copies to:

PHARMAC Board
Lead DHB CE, Pharmaceuticals
Director-General of Health – Ministry of Health
Manager Governance and Crown Entities – Ministry of Health
Principal Advisor, Crown Entity Monitoring and Appointments – Ministry of Health

Contact(s)

Sarah Fitt, Chief Executive s9(2)(a)
Alison Hill, Director Engagement and Implementation s9(2)(a)

Out of scope

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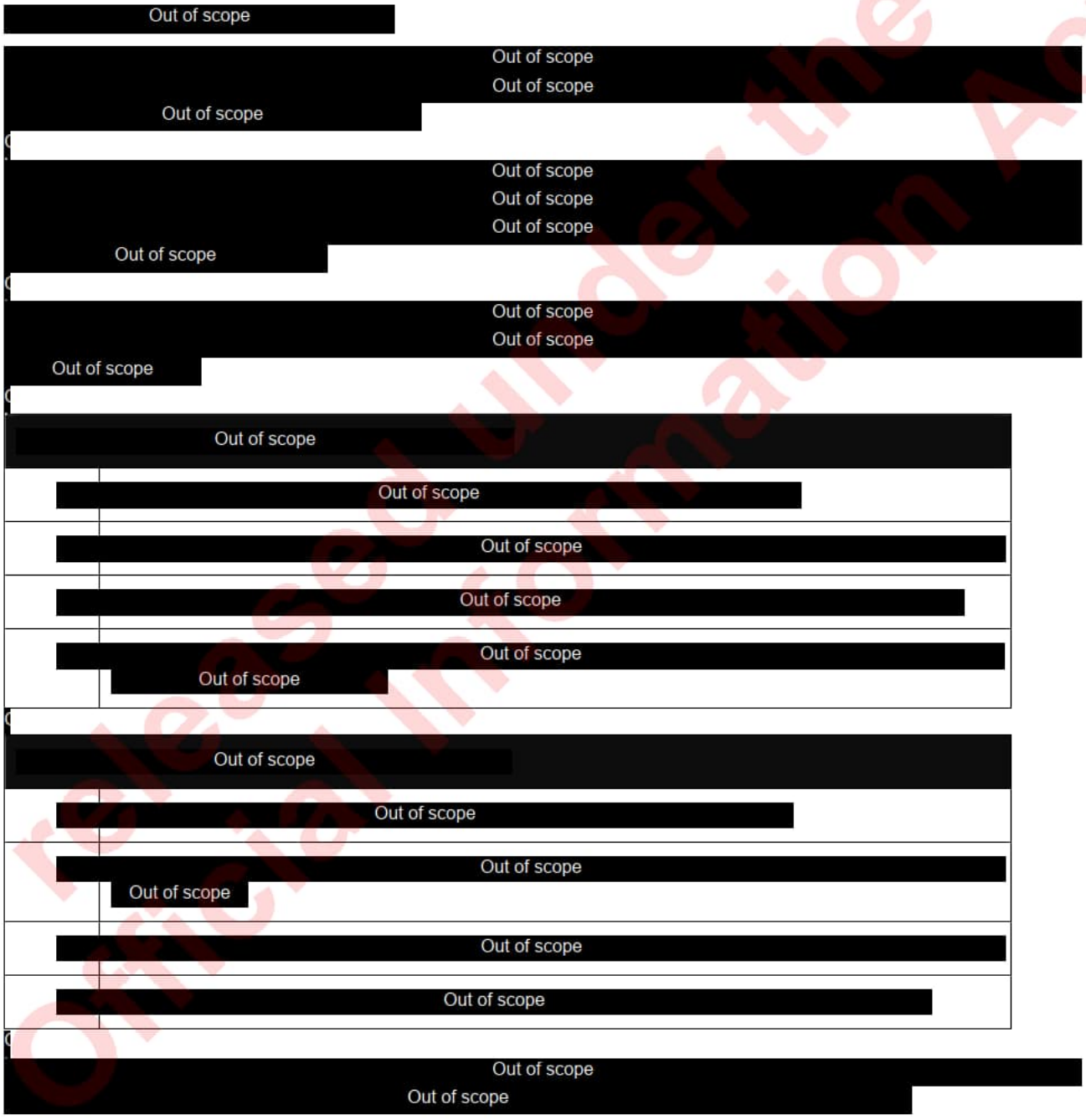
Trikafta funding application

There is continued public and media interest in Trikafta, a medicine for people with cystic fibrosis. The supplier, Vertex, has now submitted its registration application to Medsafe and has advised PHARMAC that it will be submitting a funding application for Trikafta in the coming weeks.



From: Jane Wright
Sent: Thursday, 8 July 2021 6:02 pm
To: Elisabeth Brunt
Cc: Adelia Hallett; Lizzy Cohen; Talisa Kupenga; Peter.Jane s9(2)(a) ; Fiona.Ryan s9(2)(a) ; Rachel Read
Subject: No Surprises - Trikafta

Kia ora,

This is a no surprises update for the Minister on Trikafta. Vertex has applied for its triple combination modulator therapy medicine, Trikafta, to be funded by Pharmac for people with cystic fibrosis aged 6 years and older. This makes New Zealand the first country to receive a funding application for those under the age of 12 years.

Both our Respiratory Subcommittee and our Pharmacology and Therapeutics Advisory Committee (PTAC) will assess the scientific evidence for this medicine over the coming months.

Pharmac are updating key stakeholders that a funding application arrived today, on 8 July 2021. We will issue a media release on 9 July 2021 (and share through our social media channels) confirming that we have received an application, and what the next steps are.

Please let me know if you have any questions.

Nga mihi

Jane

Jane Wright | Senior Communications Advisor, Media

PHARMAC | Te Pātaka Whaioranga | PO Box 10 254 | Level 9, 40 Mercer Street, Wellington
M: s9(2)(a) www.pharmac.govt.nz

PHARMAC
TE PĀTAKA WHAIORANGA

BRIEFING

Towards greater transparency - public release of Pharmac's Options for Investment list

Date: **22 July 2021**

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

Copies to

Hon Dr Ayesha Verrall, Associate Minister of Health
Hon Peeni Henare, Associate Minister of Health
Director General of Health
Pharmac Board
Lead DHB Chief Executive, Pharmaceuticals
Principal Advisor, Crown Entity Monitoring and Appointments, Ministry of Health

Recommendations

It is recommended you:

- **note** the contents of this briefing

Contact(s)

Sarah Fitt, Chief Executive	s9(2)(a)
Alison Hill, Director Engagement and Implementation	s9(2)(a)

[redacted] Out of scope

Publishing the ranking lists

We have created an automated list function within the Application Tracker where people can view the medicines funding proposals on Pharmac's ranking lists in alphabetical order online.

Work is underway to publicly release the ranking lists on Tuesday 27 July 2021.

We will proactively engage with media (health and national) about the lists and will notify key stakeholders when the lists are made public.

These lists change frequently when options are added, or items are removed as they are funded. This means the number of medicines or applications on the lists will continue to change.

A copy of the OFI list is attached as Appendix One. Screenshots of the automated list function within the Application Tracker are attached as Appendix Two. We note that several of these medicines have been the subject of Parliamentary petitions or media stories about patients or advocacy groups that are seeking for them to be funded.

A communications plan has been developed to manage the risks associated with publishing the lists…

Publishing our ranking lists will release a large amount of complex information to the public. If our messaging is not clear, this information could be misinterpreted. To mitigate this risk, we have developed a set of key messages that explain the lists, including how they fit together, and Pharmac's processes, which will be available on our website.

...including developing questions and answers.

We have considered the questions that are likely to be asked and have developed reactive responses to them. We expect people may want to know the cost of each individual medicine if it were to be funded. This information will not be released as it is commercial-in-confidence. Some medicines that are under assessment (such as Trikafta for cystic fibrosis, which Pharmac received an application for recently) will not be visible on any of the lists, as medicines applications are not ranked until clinical advice has been sought and our assessment process is completed.

PHARMAC
TE PĀTAKA WHAIORANGA

REPORT FOR QUARTER 4 2020/21

INCORPORATING MONTHLY REPORT FOR JULY 2021

COMMERCIAL IN CONFIDENCE

Date **5 August 2021**

Attention **Hon Andrew Little**
Minister of Health

Copies to:

Pharmac Board
Lead DHB CE, Pharmaceuticals
Director-General of Health – Ministry of Health
Manager Governance and Crown Entities – Ministry of Health
Principal Advisor, Crown Entity Monitoring and Appointments – Ministry of Health

Contact(s)

Sarah Fitt, Chief Executive s9(2)(a)
Alison Hill, Director Engagement and Implementation s9(2)(a)

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Trikafta funding application

There is continued public and media interest in Trikafta, a medicine for people with cystic fibrosis. The supplier, Vertex, has submitted its registration application to Medsafe and we have now received a funding application for Trikafta. We will be seeking expert clinical advice on the Trikafta application in late August 2021.

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Recent briefings

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- 8 July 2021 – No surprises – Trikafta funding application

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Sarah Fitt

Sarah Fitt
Chief Executive

From: Rachel Read
Sent: Friday, 24 September 2021 4:43 pm
To: Peter Jane
Subject: RE: an urgent request

Hi Peter

Lines for your briefing

Cheers Rachel

New medicines for cystic fibrosis
Pharmac has recently funded two new medicines specifically for those with cystic fibrosis:
Ivacaftor has been funded since March 2020 for the 35 New Zealanders with cystic fibrosis who have the G551D mutation (or other class III gating mutations)
Creon Micro, which is a modified-release granule formulation of pancreatic enzymes, that helps young children with cystic fibrosis digest their food more easily, has been funded since June 2020.

Other medicines funded for cystic fibrosis include:
Pancreatic enzyme
Dornase alfa – a mucolytic
Supportive care including antibiotics

Trikafta
In July 2021 Pharmac received an application from Vertex for Trikafta, a triple combination modulator therapy medicine, to be fully funded for people aged over six with cystic fibrosis. New Zealand is the first country to receive an application for those under the age of 12.
Pharmac first asked Vertex almost a year ago to submit an application and supporting clinical information. Vertex has submitted a marketing approval application to Medsafe, so Pharmac will be considering the funding application in parallel with Medsafe's assessment of the safety, quality, and efficacy of the medicine.
Pharmac is currently assessing the application, and considering clinical advice.
Trikafta could benefit approximately 400-500 people in New Zealand, if it was funded.
Vertex offers a compassionate access scheme, so some children in NZ are receiving treatment with Trikafta

Cystic Fibrosis NZ
Pharmac has a good relationship with Cystic Fibrosis NZ, meeting with them regularly

Media releases we have put out on cystic fibrosis medicines:
https://pharmac.govt.nz/news-and-resources/news/2021-07-09-media-release-application-for-cystic-fibrosis-medicine-received-by-pharmac/?type=1&page=2
https://pharmac.govt.nz/news-and-resources/news/teen-thriving-on-pharmac-funded-medicine/?type=1&page=3
https://pharmac.govt.nz/news-and-resources/news/pharmac-to-fund-new-cystic-fibrosis-medicine/?type=1&page=4

Rachel Read | Manager, Policy and Government Services | Engagement & Implementation
Pharmac | Te Pātaka Whaioranga | PO Box 10 254 | Level 9, 40 Mercer Street, Wellington
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www.pharmac.govt.nz

-----Original Message-----
From: Peter Jane <Peter.Jane s9(2)(a) >
Sent: Friday, 24 September 2021 2:03 pm
To: Rachel Read <rachel.read s9(2)(a) >
Subject: an urgent request

Hi Rachel

See the request below I am trying to change the date till Monday - but would someone be able to put together some information on treatments?

Can we please have a brief aide memoir by 4:30pm today with background on cystic fibrosis (what is it, how many people does it affect in NZ, prognosis etc)

Can we also have talking points on the treatments that are approved, the treatments that are funded and any that are in the pipeline to be approved and/or funded. Any additional comments that you think would assist the Minister would be greatly appreciated.
Comment on how our treatment and funding options compare to some of our main comparators would be useful if you have time.

Peter Jane
Principal Advisor
Crown Entity Monitoring and Appointments Ministry of Health

Email: peter.jane s9(2)(a)
Mobile: s9(2)(a)

From: Rachel Read
Sent: Tuesday, 26 October 2021 12:47 pm
To: 'Haley Ataera'
Cc: 'talisa.kupenga s9(2)(a) '; 'Adelia.Hallett s9(2)(a) '; Peter Jane; 'Fiona Ryan'; Andi Shirtcliffe; Lizzy Cohen
Subject: No Surprises - Vertex funding application for cystic fibrosis treatment
Attachments: 2021-10-27 Trikafta application progress media release.pdf

Kia ora Haley

This is a no surprises update for the Minister on Vertex's funding application for its triple combination modulator therapy, known in some countries as Trikafta, used to treat cystic fibrosis.

Our Respiratory Subcommittee has reviewed the evidence provided by Vertex and recommended (with high priority) that Pharmac fund this treatment. Later today we will publish the record of the Subcommittee's advice on our website.

Pharmac has completed its health economic assessment and using that alongside the advice from our respiratory experts, we have determined this is a medicine we would like to fund. We are adding it to our Options for Investment list.

Next month our Pharmacology and Therapeutics Advisory Committee (PTAC) will also assess all the evidence and provide additional advice to us which will help determine where the application is ranked, against all other medicines we would like to fund.

This afternoon we will be sharing this news, embargoed, with the supplier Vertex, our clinical advisory network, advocacy groups Cystic Fibrosis NZ and Trikafta for Kiwis, and selected media who have expressed an ongoing interest in this particular funding application.

Tomorrow morning at 5am we will be putting out a media release (draft attached) with a link to our website for more information. Pharmac's director of operations Lisa Williams will be Pharmac's spokesperson and available for interviews.

Please let me know if you would like any further information.

Regards Rachel

Rachel Read | Manager, Policy and Government Services | Engagement & Implementation
Pharmac | Te Pātaka Whaioranga | PO Box 10 254 | Level 9, 40 Mercer Street, Wellington
DDI: s9(2)(a) | P: +64 4 460 4990 | M: s9(2)(a)
www.pharmac.govt.nz

PHARMAC
TE PĀTAKA WHAIORANGA

Media release

27 October 2021

Cystic fibrosis medicine added to Pharmac's options for investment list

Pharmac has announced that Vertex's triple combination modulator therapy, known in some countries as Trikafta, has been added to the priority list of medicines it would like to fund.

In July this year, pharmaceutical company Vertex applied to Pharmac for its triple combination modulator therapy to be funded for people aged over 6 with cystic fibrosis.

"We've added this medicine to our options for investment list," says Pharmac's director of operations Lisa Williams. "We followed our usual process – we sought clinical advice from our Respiratory Subcommittee, and we undertook an economic health assessment.

Next month our Pharmacology and Therapeutics Advisory Committee (PTAC) will assess all the evidence and provide additional advice to us which will help determine where the application is ranked, against all other medicines we would like to fund.

"Our clinical advisers tell us if a medicine has the evidence to back up the claims made by pharmaceutical companies. Our respiratory experts have reviewed this evidence and recommended that Pharmac fund Vertex's triple combination modulator therapy. They gave this a high priority," says Lisa. "We've published their clinical advice on our website. Using this information and our own economic health assessment, we have determined this is a medicine we would like to fund."

"Before it could be funded by Pharmac, we would need a deal with the supplier and enough money in our budget to fund it. We would also need to ask New Zealanders what they think before we made any final decision to fund this new medicine."

"We can't shy away from the fact that it's a very expensive medicine," says Lisa. "While Pharmac can negotiate some of the best prices for medicines in the world, when faced with a medicine which has a patent with such a long patent life, such negotiations can be challenging."

"As of today, there are 114 applications on our options for investment (OFI) list, and we would like to fund them all. They would all benefit patients if funded. This means we need to make some difficult choices and why prioritisation of these medicines is incredibly important."

Ends

Please email media@pharmac.govt.nz or call 021863342 if you would like to know more.

Learn more:

- Cystic fibrosis is an inherited life-threatening disorder that damages the lungs and digestive system.

- Vertex's triple combination modulator therapy is called Trikafta in some countries, and Kaftrio in others. It is a combination of three different medicines - elexacaftor, tezacaftor and ivacaftor.
- Vertex applied to New Zealand's medicines and medical devices regulator, Medsafe, for market approval of their medicine in June 2021. Medsafe's website shows they issued their first request for information to Vertex on 15 October 2021. It has been granted priority assessment status https://www.medsafe.govt.nz/regulatory/ProductDetail.asp?ID=22462.

From: Allanah Andrews
Sent: Tuesday, 22 February 2022 12:09 pm
To: Haley Ataera; Adelia Hallett
Cc: Peter.Jane s9(2)(a) ; Carol Morris; Andi.Shirtcliffe s9(2)(a) ; Talisa Kupenga; Allison Bennett; Jane Wright
Subject: No Surprises - Trikafta application update

Kia ora Hayley,

This is a no surprises update for the Minister on Vertex's funding application for their triple combination modulator therapy (branded Trikafta), used to treat cystic fibrosis.

In July last year, pharmaceutical company Vertex applied to Pharmac for Trikafta to be funded for people aged over 6 with cystic fibrosis with at least one F508del mutation in the cystic fibrosis transmembrane conductance regulator (CFTR) gene.

PTAC has reviewed the evidence provided by Vertex and recommended (with medium priority) that Pharmac fund the treatment for those aged over 12. They have deferred making a recommendation regarding its funding for those between the ages of 6 and 11 until further evidence has been provided. We will publish the record of PTAC's advice on our website on Wednesday 23 February.

PTAC's recommendation is different to Pharmac's Respiratory Specialist Advisory Committee's recommendation received in August 2021. The Respiratory Committee recommended, with high priority, that Pharmac fund Trikafta for all those 6 years of age and older.

We already determined last year that Trikafta is a medicine we would like to fund. We'll now use the recommendations from both our Respiratory Advisory Committee and PTAC, to review the ranking of funding for all those 6 years of age and older on our Options for Investment (OFI) list and at the same time we'll add and rank a new option – funding for those aged 12 and over.

On Wednesday 23 February, we intend to put out a media release with a link to our website for more information. Pharmac's director of operations Lisa Williams will be Pharmac's spokesperson and available for interviews.

While we are reconfirming that Pharmac would like to fund Trikafta, the news is likely to be met with negative reaction from media and particularly consumer groups who are advocating for this medicine to be funded as soon as possible and will be concerned that this will further delay a funding decision.

Please let me know if you would like any further information.

Ngā mihi / Warm Regards,

Allanah Andrews (she/her) | Manager, Policy and Government Services
Te Pātaka Whaioranga | Pharmac
PO Box 10-254, Wellington 6140 | Level 9, 40 Mercer Street, Wellington 6011
P: s9(2)(a) | www.pharmac.govt.nz