Thursday 12 January 2023

Email to:**consult@pharmac.govt.nz**

**PROPOSAL TO FUND ELEXACAFTOR WITH TEZACAFTOR AND IVACAFTOR (TRIKAFTA) FOR PEOPLE WIH CYSTIC FIBROSIS**

Thank you for the opportunity to provide feedback on the proposal to fund Trikafta for people with cystic fibrosis (CF) aged 6 years and over from 1 April 2023.

Rare Disorders NZ strongly support:

* Pharmac’s proposal to fund Trikafta for people with CF aged 6 years and over; and
* The proposed eligibility criteria for access to Trikafta as set out in Pharmac’s proposal.

Rare Disorders NZ have been long-time advocates for access to medicines for Rare Disorders including Cystic fibrosis. For the purpose of this submission, we will keep it to CF, an area of significant unmet need.

It is important to reference the advocacy as it has taken a significant toll on patients, their loved ones, and the rare disorder community in the time that it has taken to get to this point.

***Benefits of and Support for Funding Trikafta***

**Trikafta is the first medicine to treat the cause of CF for the majority of people with this devastating and life-limiting condition. Funding Trikafta will:**

* Bring life-changing benefits to more than 350 people with CF in Aotearoa New Zealand and enable them to live almost normal lives.
* Help to prevent young children from experiencing significant ill-health and decline.
* Substantially extend the lives of people with CF in New Zealand, providing an estimated additional 27 years of full health and almost doubling their life expectancy from the current 31 years of age.
* Provide relief from the burden of anxiety, depression, and grief that many people with CF and their families currently face, and give them a future to look forward to.
* Provide significant benefits to New Zealand’s health system, with a substantial reduction in medical treatment, hospitalisations, and lung transplants.

**There is overwhelming support for funding Trikafta in New Zealand as shown by:**

* Extensive media coverage over the last two years; and
* A petition signed by more than 43,400 people. Given there are around 570 people with CF in New Zealand, this is a major achievement.

***Every Kiwi with CF needs access to a life-changing medicine
like Trikafta***

While Trikafta will benefit the majority of people with CF in New Zealand there are some for whom it will not be a suitable option, while others may not be able to take Trikafta and will need access to an alternative therapy. Some people with CF will need access to Kalydeco and Symdeko – CF therapies that are also made by Vertex.

**Pharmac already funds Kalydeco for people with CF who have certain gene mutations and is currently considering an application to widen access to include additional mutations:**

* Pharmac’s Respiratory Advisory Committee has recommended a high priority for funding the widening of access to Kalydeco to additional CF mutations; and
* There is significant crossover between the mutations included in the proposed eligibility criteria for access to Trikafta and the widening of access to Kalydeco.

Symdeko received consent for use in New Zealand from Medsafe in January 2022 and would provide an alternative for some people with CF who may be unable to take Trikafta.

To ensure equity and fairness of treatment for people with CF in Aotearoa New Zealand every person with CF needs access to a CF therapy that works for them from 1 April 2023.

**We therefore request that the final agreement between Pharmac and Vertex for Trikafta includes:**

* The widening of access to Kalydeco; and
* Access to Symdeko for people with CF who may be unable to take Trikafta.

***Conclusion***

Thank you for the opportunity to provide feedback on the proposal to fund Trikafta for people with CF aged 6 years and over from 1 April 2023.

We look forward to a positive decision in February and for people with CF to be able to access these life-changing medicines from 1 April 2023.

Nga mihi

Michelle Arrowsmith

Chief Executive Officer, Rare Disorders NZ

On behalf of Rare Disorders NZ