MEDIA RELEASE
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Cystic fibrosis community disappointed with PTAC recommendations

New Zealand's cystic fibrosis community received disappointing news today with a recommendation to Pharmac that reduces the priority for funding Trikafta from **high to medium** for 12 and over, and the decision be **deferred** for those under the age of 12, pending the availability of further data.

Trikafta, the drug Pharmac called "paradigm-shifting", treats the cause for most people with cystic fibrosis.

In October last year, the CF community received the welcome news they'd been waiting for. A public announcement from Pharmac confirmed that its expert clinical advisers recommended a high priority for funding Trikafta for people with cystic fibrosis aged 6 years and older.

Pharmac added Trikafta to the list of medicines it wants to fund and sought further advice from its Pharmacology and Therapeutics Advisory Committee (PTAC) on how Trikafta should be ranked against other medicines.

Today Pharmac published the minutes that include PTAC's recommendations. These recommendations are inconsistent with those from the Respiratory Subcommittee and this has now created real uncertainty about the priority and what it will mean for the cystic fibrosis community.

Lisa Burns, Chief Executive of Cystic Fibrosis NZ, says, "How do we support families affected by these recommendations, knowing that this will have a devastating impact on the hope that's been built up over the past two years?".

"PTAC's recommendation to defer funding Trikafta for age 6 to 11 years is very concerning," said Lisa. CFNZ is concerned that children under the age of 12 may miss out, taking away their opportunity for early treatment and to reduce long term damage. Many already live with significant ill health, demanding and time-consuming daily regimes of lung clearance and medication, and multiple hospital admissions for IV antibiotics each year.

"We are devastated for our CF community, there are no words that will provide comfort when they hear this news," says Lisa. "Life with cystic fibrosis is a rollercoaster – sudden infections or health complications shatter hopes and plans without warning, and these recommendations just add to their worry about the future".

The harsh realities of life with cystic fibrosis (CF) mean that, at just 11 years old, children like Brett have already had frequent hospital admissions fighting life-threatening infections.

"Night-time is always hard for Brett," his mum Penny says. "He wants to go to sleep but he worries about dying too young and it often keeps him awake. He just wants the same opportunities as everyone else, but he knows about his shorter life expectancy, and he knows that his life is going to be a hard road. It truly breaks my heart".

Trikafta is now funded in 25 countries around the world, meaning New Zealand is years behind in providing life-saving treatments that would make a real difference for Kiwis living with cystic fibrosis. Pharmac has been provided with more data and evidence than any other country to support the application, making it hard to understand the reasoning behind PTAC's recommendations.

Trikafta has been assessed and granted regulatory approval by Medsafe for use in New Zealand for those aged 6 years and older. It would provide life-changing benefits to Kiwis with CF, like Brett and hundreds of others, by substantially improving their quality of life and life expectancy. It would let them live almost normal lives.

Real world data recently published by Vertex from more than 16,000 CF patients in the US reinforces the effectiveness of Trikafta:

- 87% reduction in risk of lung transplant*
- 77% fewer serious lung infections**
- 74% reduction in risk of death*

"The lives of people with cystic fibrosis matter. Investing in Trikafta would also deliver substantial benefits to the New Zealand health system, through significant reductions in medical treatment, hospitalisations and lung transplants" says Lisa.

Pharmac has made much of how expensive Trikafta is. Cystic Fibrosis NZ acknowledges the difficult decisions Pharmac must make when deciding which medicines to fund from its limited budget, however price needs to be put into perspective. Pharmac prides itself on achieving some of the best commercial prices for pharmaceuticals in the world and we know that it will not pay the full retail price for Trikafta.

Speaking on behalf of the whole CF community Lisa says "We need to understand the pathway forward now. We want to work with Pharmac and urge them to re-confirm their October commitment that Trikafta has a high priority ranking for funding for all who will benefit, aged 6 years and older".

* Unadjusted estimate relative to historical 2019 U.S. Cystic Fibrosis Foundation registry data for patients older than 12 with at least one copy of F508del mutation
** Relative to 12-month period prior to TRIKAFTA treatment initiation
Data from observational post-authorization safety study on >16,000 TRIKAFTA-treated patients with mean of ~9 months of exposure, from U.S Cystic Fibrosis Foundation patient registry.

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For more detailed information please refer to the Cystic Fibrosis NZ media kit.