The Paediatric Society of New Zealand
Te Kāhui Mātai Arotamariki o Aotearoa

NZCYCN
New Zealand Child & Youth Clinical Network

11 Feb 2025

Submission: Proposal to widen access to Trikafta and Kalydeco and fund Alyftrek for the treatment of children and adults with cystic fibrosis

The Respiratory and Sleep Clinical Network of Te Kāhui Mātai Arotamariki o Aotearoa | The Paediatric Society of New Zealand welcomes Pharmac's proposal to widen access to existing treatments and fund new treatments for people with cystic fibrosis from 1 April 2026 through a provisional agreement with Vertex Pharmaceuticals (Australia) Pty Ltd.

We strongly support the proposal to:

- Widen access to Trikafta for all people with eligible mutations
- Widen access to Kalydeco for all people with eligible mutations; and
- Fund Alyftrek for all people with eligible mutations.

We have already seen the impact that modulator therapy has had on our school-age children, teenagers and adults with cystic fibrosis, leading to significantly improved lung function, fewer infective respiratory exacerbations, and fewer hospitalisations in the longer term, improving the disease progression trajectory. Unsurprisingly, this has hugely improved the quality of life, allowing individuals to participate in school, higher education and work opportunities. As we have seen from international experiences, this will lead to an increased life expectancy.

Extending access to younger children with eligible mutations will enable improvement of the disease progression trajectory further by treating those prior to developing lung damage. We would now expect them to have a longer and healthier life with a future to look forward to.

Criteria for Access

We generally agree with the proposed criteria for access, but have a few reservations which we would like Pharmac to consider:

- We would like Pharmac to continue the link to the list of eligible mutations for each medicine to the United States Food and Drug Administration (FDA) prescribing. This is rigorously maintained up to date as information comes through. Changing it to a Medsafe list, given often resourcing constraints with that organisation, may mean that we will fall behind those who have eligibility to receive these medications internationally, but will be delayed in New Zealand. This will only impact a very small number of individuals, but often those of particular ethnic groups who have less common genetic mutations, and for those few individuals, it will make a substantial difference to their lives.

- There are also a very small number of individuals who have a definitive diagnosis of cystic fibrosis but in whom their original sweat chloride is less than 60 mmol/L - often in the upper 50s. Internationally, it is now recognised that there is suspicion of cystic fibrosis in individuals with a sweat chloride > 30 mmol/L. There is some difference in the criteria for each of the three medications.

For Ivacaftor, it is necessary that:

- Patient has been diagnosed with cystic fibrosis; **and**
- Patient must have at least one mutation on the list of CFTR mutations that produce CFTR protein and are responsive to ivacaftor; **and**
- Patient must have a sweat chloride value of at least 60mmol/L by quantitative pilocarpine iontophoresis or by Macroduct sweat collection system.

For Trikafta and Alyftrek it is necessary that:

- Patient has been diagnosed with cystic fibrosis; **and**
- Either:
    - Patient has two cystic fibrosis causing mutations in the CFTR regulator gene (one from each parental allele); or
    - Patient has a sweat chloride level of at least 60mmol/L by quantitative pilocarpine iontophoresis or by Macroduct sweat collection system.

It would be helpful if these criteria were the same, and the latter is more in keeping with our clinical experience.

However, we are generally very supportive of Pharmac's proposal to widen access to Trikafta and Kalydeco, and to fund Alyftrek from 1 April 2026. It will align New Zealand with 30 other countries that already offer Trikafta from the age of two, and among the first to have access to Alyftrek for those aged 6 years and over.

Thank you for the opportunity to submit on this proposal.

Ngā mihi,

Co-Chairs, and on behalf of:

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