26th April 2024

RECOMMENDED: Trikafta for 2 - 5 year olds

Today we can celebrate. We have received the great news that the existing listing of Trikafta will be expanded to include 2– 5-year-olds with at least one F508del mutation on the cystic fibrosis transmembrane conductance regulator (CFTR) gene.

This is fantastic news because we know that there is a significant benefit to commencing therapies early to prevent irreparable damage. With this listing, 130 children will gain access to a modulator for the first time, while 290 children will have access to three modulator therapies. This represents significant progress in ensuring universal access to necessary therapies.

1. Your voices have been heard.

It is clear that the community support and advocacy have backed this outcome. Thank you to everyone who made a submission, including our Federation Members. It is evident, time and time again, that when we unite, we can help drive progress.

The next hurdle is knowing when Trikafta will be subsidised by the Pharmaceutical Benefits Scheme (PBS) so that it can be accessed by those in the 2-5-year age group. We hope that there is no delay and we call on all stakeholders to please accelerate this listing, do not delay access for these little ones.

I reflect that we are still waiting and hoping for the PBS listing for Kalydeco. It felt like a Christmas miracle when we had the news of that announcement in December 2023 for 4 months and older including additional gene mutations took place. Now more than four months later people are still waiting to access it. The benefit of recommending Kalydeco has not (yet) been realised because the people who need it cannot access it! This must change. **We need this listed on the PBS URGENTLY.** The decision today in recommending Trikafta for 2-5-year-olds also must not experience delays in being listed on the PBS.

We are continuing to liaise with all stakeholders in the hope of minimising the delays between PBAC recommendations and PBS listings. Currently, it is taking too long.

I acknowledge that this news today may be difficult for people with rare mutations and people who do not respond well to modulators. If that is you, then please be assured that our work includes you as well. While today may not directly benefit you, it does demonstrate an expansion of the listing of Trikafta, and this is good progress for the whole community. Hopefully, we will see a continuation of therapies being expanded for everyone who will benefit from them.

As your national peak body, we represent all people living with cystic fibrosis and we are relentless in ensuring that everyone with cystic fibrosis has access to the therapies we need.

Today we can celebrate a great outcome and for those who will not directly benefit from it, I hope you can see a ray of hope on the horizon for the outcomes you need as well.

To read the PBAC web outcome, head to the website and search ELEXACAFTOR WITH TEZACAFTORAND WITH IVACAFTOR, AND IVACAFTOR.

PBAC WEB OUTCOMES

Warm Regards,

Jo Armstrong
CEO
Cystic Fibrosis Australia